THE ROSEN LAW FIRM, P.A.
Laurence M. Rosen
609 W. South Orange Avenue, Suite 2P
South Orange, NJ 07079
Telephone: (973) 313-1887
Facsimile: (973) 833-0399
Email: lrosen@rosenlegal.com

Counsel for Plaintiff

UNITED STATES DISTRICT COURT
DISTRICT OF NEW JERSEY

NED KIM, derivatively on behalf of PTC THERAPEUTICS, INC., Plaintiff, vs. STUART W. PELTZ, SHANE KOVACS, MICHAEL SCHMERTZLER, RICHARD ALDRICH, ALLAN JACOBSON, ADAM KOPPEL, MICHAEL KRANDA, C. GEOFFREY MCDONOUGH, RONALD C. RENAUD, JR., DAVID P. SOUTHWELL, JEROME ZELDIS, and GLENN D. STEELE, JR., Defendants, and PTC THERAPEUTICS, INC., Nominal Defendant.	Case No. 17-cv-8062 **DEMAND FOR JURY TRIAL**

VERIFIED SHAREHOLDER DERIVATIVE COMPLAINT

INTRODUCTION

Plaintiff Ned Kim ("Plaintiff"), by his undersigned attorneys, derivatively and on behalf of Nominal Defendant PTC Therapeutics, Inc. ("PTC" or the "Company"), files this Verified Shareholder Derivative Complaint against Individual Defendants Stuart W. Peltz, Shane Kovacs,

Michael Schmertzler, Richard Aldrich, Allan Jacobson, Adam Koppel, Michael Kranda, C. Geoffrey McDonough, Ronald C. Renaud, Jr., David P. Southwell, Jerome Zeldis, and Glenn D. Steele, Jr. (collectively, the "Individual Defendants" and together with PTC, the "Defendants") for breaches of their fiduciary duties as directors and/or officers of PTC, unjust enrichment, abuse of control, gross mismanagement, waste of corporate assets, and violations of Section 14(a) of the Securities Exchange Act of 1934 (the "Exchange Act"). As for his complaint against the Defendants, Plaintiff alleges the following based upon personal knowledge as to himself and his own acts, and information and belief as to all other matters, based upon, *inter alia*, the investigation conducted by and through Plaintiff's attorneys, which included, among other things, a review of the Defendants' public documents, conference calls and announcements made by Defendants, United States Securities and Exchange Commission ("SEC") filings, wire and press releases published by and regarding PTC, legal filings, news reports, securities analysts' reports and advisories about the Company, and information readily obtainable on the Internet. Plaintiff believes that substantial evidentiary support will exist for the allegations set forth herein after a reasonable opportunity for discovery.

NATURE OF THE ACTION

1. This is a shareholder derivative action that seeks to remedy wrongdoing committed by PTC's directors and officers starting on November 6, 2014 and through the present (the "Relevant Period").

2. PTC is a biopharmaceutical company that focuses on the discovery, development, and commercialization of orally administered, small molecule therapeutics which target an area of RNA biology the Company refers to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA is copied

from DNA through the transcription process. The Company's pipeline addresses multiple therapeutic areas, including oncology, rare disorders, and infectious diseases.

3. The Company's lead product is Translarna (ataluren), which PTC primarily seeks to commercialize for the treatment of Duchenne muscular dystrophy ("DMD"), among other diseases.

4. Prior to and during the Relevant Period, the Company conducted multiple clinical trials to prove the efficacy of Translarna with the ultimate goal of getting it approved by the FDA for treatment of all forms of DMD. The most important of these studies during the Relevant Period was the Company's Phase 3 clinical trial[1] for Translarna for the treatment of an extremely rare genetic disorder called nonsense mutation Duchenne muscular dystrophy ("nmDMD"), known as the "ACT DMD," or the "Ataluren Confirmatory Trial." The results of this trial would be used by the Company in its New Drug Application ("NDA") for Translarna which would need to be approved by the U.S. Food and Drug Administration (the "FDA") prior to PTC being able to market and sell Translarna in the U.S.

5. However, the ACT DMD Phase 3 trial was plagued with issues that the Individual Defendants kept hidden, including failures to meet self-determined criteria for success, post hoc manipulation of data that would prove to be unusable, and reliance on data samples that were hand-picked to create the illusion of success. Ultimately, the Company's clinical trials were unable to show a statistically significant treatment efficacy in nmDMD patients.

6. On February 23, 2016, the Company announced that it had received a Refuse-to-File ("RTF") letter from the FDA regarding the Company's NDA for Translarna, which was completed by the Company and fully submitted in January 2016 (the "2016 NDA"). A Refuse-to-

[1] Unless otherwise noted, references to "Phase 3 clinical trial," "Phase 3 trial," "ACT DMD," and "ACT DMD Phase 3 trial" throughout are coterminous and refer to the same clinical trial for Translarna.

File letter communicates summary rejection by the FDA. The RTF letter informed PTC that their 2016 NDA for Translarna was summarily rejected as a result of a series of facially obvious deficiencies, and that due to deficiencies that are not a matter of interpretation, the FDA will not undertake even the standard initial step of filing PTC's 2016 NDA for Translarna.

7. On this news, the price per share of PTC stock fell $17.42 per share, or approximately 61.6%, from the previous day's closing price to close at $10.84 on February 23, 2016.

8. The Company appealed the FDA's decision, and the Company announced on October 17, 2016 that the FDA had denied its appeal.

9. On March 6, 2017, the Company announced that the FDA had acknowledged a filing-over-protest of the NDA for Translarna, allowing for review of the NDA despite the FDA finding that it was facially insufficient to support substantive review. FDA regulations allow for companies to have their NDA's filed and reviewed against the recommendations of the FDA following receipt of a RTF determination.

10. On June 6, 2017, the Company announced in a press release that the FDA had notified the Company of a meeting with the Peripheral and Central Nervous Systems Drugs Advisory Committee ("the PCNSDAC") scheduled for September 28, 2017 to review the filed-over-protest NDA for Translarna (the "Advisory Committee Meeting"). The press release also noted that the FDA had set a goal date of October 24, 2017 for completion of its review of the NDA for Translarna.

11. On September 26, 2017, the PCNSDAC published briefing materials in advance of the Advisory Committee Meeting, which reaffirmed the failures of the NDA for Translarna,

however in much greater detail and to further extent. In fact, as noted by the health and medicine news outlet *STAT*, "Translarna was rather robustly savaged" in the review.

12. On this news, the price per share of PTC stock fell $2.70 per share, or approximately 13.8%, from the previous day's closing price to close at $16.81 on September 26, 2017.

13. On September 28, 2017, 10 of the 11 members of the PCNSDAC

14. During the Relevant Period, the Individual Defendants personally made and/or caused the Company to make a series of materially false and/or misleading statements regarding the Company's business, operations, prospects and legal compliance. Specifically, the Individual Defendants willfully or recklessly made and/or caused the Company to make false and/or misleading statements and/or omissions of material fact that failed to disclose:

(1) The substantial risk that the NDA submission for Translarna would be rejected by the FDA as facially insufficient and that the ACT DMD trial would not meet its primary clinical endpoints, thus resulting in the FDA refusing to file the 2016 NDA for substantive review and only ultimately reviewing it because it was filed-over-protest by the Company;

(2) PTC would be required by the FDA to demonstrate the efficacy of Translarna more sufficiently than PTC had done in the Phase 2b trials, and the ACT DMD trial's portrayal as "confirmatory" was materially misleading;

(3) The substantial likelihood that the 2016 NDA would not be reviewed at all by the FDA if the results of the ACT DMD trial did not "confirm" Translarna's efficacy as demonstrated purportedly by the Phase 2b trial's results;

(4) There was just as much of a risk of failure in the design of the ACT DMD trial as was present in the Phase 2b trial, any risks of negative outcomes were not lessened by the

design of the ACT DMD, and there was no basis to believe that the outcomes of the ACT DMD trial would positively demonstrate efficacy as determined by the trial's primary clinical endpoints;

(5) The Phase 3 trial results were less supportive of a finding of efficacy than the results drawn from the Phase 2b trial—and thus did not "confirm" the benefit for DMD patients in using Translarna—and this was facially insufficient to support substantive review by the FDA;

(6) The claimed efficacy of Translarna was supported by meta-analyses results that were only applicable to a small subgroup of nmDMD patients and not pre-specified in PTC's statistical analysis plan, meaning that the meta-analyses would be facially insufficient to form a complete application that the FDA would file;

(7) The FDA would consider the Company's reliance on the 300-400 meter subgroup as the main analysis to be a post-hoc adjustment, and that the Company's ultimate submission discarded data from a majority of the Phase 3 trial's patients, thus rendering inadequate on its face the 2016 NDA submission;

(8) The risk of the 2016 NDA being facially insufficient to support an approval for Translarna's broad label use for all nmDMD patients was underrepresented, and that the Individual Defendants in fact knew that only showing statistical significance for a small subgroup and in meta-analyses would be facially insufficient to support a complete application that the FDA would review;

(9) The statements made by the Defendants suggesting that the Company's gathered data was sufficient to support substantive review or FDA approval were contradicted by additional efficacy requirements relayed to PTC in discussions with the FDA after PTC

received the RTF letter for its 2011 NDA (as defined herein) and in guidance provided to the Company from the FDA regarding the development of drugs for the treatment of DMD;

(10) The primary clinical endpoints and other intent-to-treat ("ITT") analyses of the ACT DMD trial were not met, and thus the trial did not demonstrate to a statistically significant level a clinically meaningful benefit of treatment;

(11) Ultimately, the ACT DMD trial's data would be facially insufficient to form a complete application that would be reviewed by the FDA without the Company filing it over protest; and

(12) The Company failed to maintain internal controls.

15. As a result of the foregoing, the Company's public statements were materially false and misleading at all relevant times. The Individual Defendants failed to correct and/or caused the Company to fail to correct these false and/or misleading statements and/or omissions of material fact, rendering them personally liable to the Company for breaching their fiduciary duties.

16. Moreover, in breach of their fiduciary duties the Individual Defendants failed to maintain internal controls.

17. In light of the Individual Defendants' misconduct, which has subjected the Company, its Chief Executive Officer ("CEO"), and its former Chief Financial Officer ("CFO") to being named as defendants in a consolidated federal securities fraud class action lawsuit pending in this Court (the "Securities Class Action"), the need to undertake internal investigations, the need to implement adequate internal controls, the losses from the waste of corporate assets, the losses due to the unjust enrichment of the Individual Defendants who were improperly over-compensated

by the Company and/or who benefitted from the wrongdoing alleged herein, the Company will have to expend many millions of dollars.

18. In light of the breaches of fiduciary duty engaged in by the Individual Defendants, most of whom are the Company's current directors, their collective engagement in fraud, the substantial likelihood of the directors' liability in this derivative action and the Company's CEO's and former CFO's liability in the Securities Class Action, their being beholden to each other, their longstanding business and personal relationships with each other, and their not being disinterested and/or independent directors, a majority of PTC's Board of Directors (the "Board") cannot consider a demand to commence litigation against themselves on behalf of the Company with the requisite level of disinterestedness and independence.

JURISDICTION AND VENUE

19. This Court has subject matter jurisdiction pursuant to 28 U.S.C. § 1331 because Plaintiff's claims raise a federal question under Section 14(a) of the Exchange Act, 15 U.S.C. § 78n(a)(1) and Rule 14a-9 of the Exchange Act, 17 C.F.R. § 240.14a-9, and raise a federal question pertaining to the claims made in the Securities Class Action based on violations of the Exchange Act.

20. This Court has supplemental jurisdiction over Plaintiff's state law claims pursuant to 28 U.S.C. § 1367(a).

21. This derivative action is not a collusive action to confer jurisdiction on a court of the United States that it would not otherwise have.

22. Venue is proper in this District because a substantial portion of the transactions and wrongs complained of herein occurred in this District, one or more of the Defendants either resides

or maintains executive offices in this District, and the Defendants have received substantial compensation in this district by engaging in numerous activities that had an effect in this District.

PARTIES

Plaintiff

23. Plaintiff is a current shareholder of PTC. Plaintiff has continuously held PTC common stock at all relevant times.

Nominal Defendant PTC

24. PTC is a Delaware corporation with its principal executive offices at 100 Corporate Court, South Plainfield, New Jersey 07080. PTC's shares trade on the NASDAQ Global Select Market ("NASDAQ") under the ticker symbol "PTCT."

Defendant Peltz

25. Defendant Stuart W. Peltz ("Peltz") has served as the Company's CEO and as a Company director since the Company's inception in 1998. According to the Company's Schedule 14A filed with the SEC on April 28, 2015 (the "2015 Proxy Statement), as of April 20, 2015, Defendant Peltz beneficially owned 377,896 shares of the Company's common stock, which represented 1.1% of the Company's outstanding common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, Peltz owned over $26.2 million worth of PTC stock.

26. For the fiscal year ended December 31, 2015, Defendant Peltz received $10,518,723 in compensation from the Company. This included $600,000 in salary, $9,600,480 in option awards, $310,000 in non-equity incentive plan compensation, and $8,243 in all other compensation.

27. The Company's 2015 Proxy Statement stated the following about Defendant Peltz:

> Dr. Peltz is a co-founder of our company and has served as our Chief Executive Officer and a member of our Board since our inception in 1998. He also serves as a director of PTC Therapeutics International Limited, our international headquarters and indirect wholly-owned subsidiary, and as a director of one of our international subsidiary boards. Dr. Peltz is a recognized scientific leader in RNA biology in the area of post-transcriptional control processes involving mRNA turnover and translation, with more than 30 years of research and over 100 publications in this area. Prior to founding our company, Dr. Peltz was a Professor in the Department of Molecular Genetics & Microbiology at the Robert Wood Johnson Medical School, Rutgers University. Dr. Peltz has received a number of business and scientific awards, including election as a Fellow of the American Academy for the Advancement of Science in 2010, recipient of the Dr. Sol J. Barer Award for Vision Innovation and Leadership in 2014 and recognition as PharmaVoice's 100 Most Inspiring People in 2009. He is a member of the board of directors for the Biotechnology Industry Organization (BIO) and serves on BIO's Emerging Companies Section Governing Board. Dr. Peltz also serves as the Chairman of the BioNJ Board of Trustees. Dr. Peltz received a Ph.D. from the McArdle Laboratory for Cancer Research at the University of Wisconsin. We believe that Dr. Peltz is qualified to serve on our Board because of his extensive executive leadership experience, many years of service as one of our directors and our Chief Executive Officer and extensive knowledge of our company and industry.

28. During the period of time when the Company materially misstated information to keep the stock price inflated, and before the scheme was exposed, Defendant Peltz made the following sales of Company stock (and made no purchases of Company stock). On March 9, 2015, Defendant Peltz sold 47,201 shares of Company stock for $71.47 per share. On March 23, 2015, Defendant Peltz sold 47,200 shares of Company stock for $69.77 per share. On June 8, 2015, Defendant Peltz sold 73,700 shares of Company stock for $55.14 per share. On June 9, 2015, Defendant Peltz sold 26,300 shares of Company stock for $53.61 per share. Thus, before the fraud was exposed, he sold 194,401 Company shares on inside information, for which he received over $12.1 million. His insider sales, made with knowledge of material non-public information before the material misstatements and omissions were exposed, demonstrate his motive in facilitating and participating in the fraud.

29. Defendant Peltz's domestic partner, Ellen Welch, is employed by the Company as its Senior Vice President, Biology.

30. Defendant Peltz's brother is a principal at RSM US LLP, a provider of tax, audit, and consulting services that PTC engaged for IT, tax and audit services with respect to the Company's 401(k) plan for fees totaling approximately $263,000. The Company notes in its Schedule 14A filed with the SEC on April 28, 2017 (the "2017 Proxy Statement") that PTC "ha[s], and anticipate that we will continue to, engage[d] RSM for these types of services during 2017."

Defendant Kovacs

31. Defendant Shane Kovacs ("Kovacs") served as the Company's Executive Vice President, CFO from June 2013 to June 2017. Although the Company does not provide Defendant Kovacs' beneficial ownership of PTC stock in its filings with the SEC until a date after the Company's false and misleading statements and omissions of material fact were disclosed, Form 4's filed with the SEC indicate that during at least some points of the Relevant Period, Defendant Kovacs beneficially owned at least tens of thousands of Company shares.

32. For the fiscal year ended December 31, 2015, Defendant Kovacs received $3,357,987 in compensation from the Company. This included $417,000 in salary, $2,739,337 in option awards, $187,650 in non-equity incentive plan compensation, and $14,000 in all other compensation.

33. The Company's 2015 Proxy Statement stated the following about Defendant Kovacs:

> Mr. Kovacs has served as our Executive Vice President, Chief Financial Officer and since June 2013 and has also served as our Head of Corporate Development since January 2015. He also serves as a director of PTC Therapeutics International Limited, our international headquarters and indirect wholly-owned subsidiary, and as an officer and director of many of our international subsidiary boards. Prior to joining us, Mr. Kovacs served in positions of increasing responsibility at Credit Suisse, an investment banking firm, from March 2004

to May 2013, including most recently as a Managing Director. From July 2002 to March 2004, Mr. Kovacs served as an associate at National Bank Financial, a diversified financial services firm. Mr. Kovacs received a B.Eng. and a B.S. in Chemical Engineering and Life Sciences from Queen's University and an M.B.A. from the University of Western Ontario. Mr. Kovacs is a Chartered Financial Analyst.

34. During the period of time when the Company materially misstated information to keep the stock price inflated, and before the scheme was exposed, Defendant Kovacs made the following sale of Company stock (and made no purchases of Company stock). On May 22, 2015, Defendant Kovacs sold 82,491 shares of Company stock for $55.59 per share. Thus, before the fraud was exposed, he sold 82,491 Company shares on inside information, for which he received over $4.5 million. His insider sale, made with knowledge of material non-public information before the material misstatements and omissions were exposed, demonstrates his motive in facilitating and participating in the fraud.

Defendant Schmertzler

35. Defendant Michael Schmertzler ("Schmertzler") has served as a Company director since 2001 and as Chair of the Board since 2004. He also serves as Chair of the Nominating and Corporate Governance Committee and as a member of the Compensation Committee. According to the 2015 Proxy Statement, as of April 20, 2015, Defendant Schmertzler beneficially owned 2,538,924 shares of the Company's common stock, which represented 7.5% of the Company's outstanding common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, Schmertzler owned over $176.3 million worth of PTC stock.

36. For the fiscal year ended December 31, 2015, Defendant Schmertzler received $770,602 worth of compensation from the Company. This included $51,000 in fees earned or cash paid and $719,602 in option awards.

37. The Company's 2015 Proxy Statement stated the following about Defendant Schmertzler:

> Mr. Schmertzler has served as a member of our Board since August 2001, as our Chair of the Board since November 2004 and as a director of our UK subsidiary since 2012. Since 2001, Mr. Schmertzler has served as a Managing Director of Aries Advisors, LLC, the sub-advisor to Credit Suisse First Boston Equity Partners, L.P., a private equity fund, and the Chair of the investment committee. From 1997 to 2001, Mr. Schmertzler was Co-Head of United States and Canadian Private Equity at Credit Suisse First Boston, an investment banking firm. Prior to 1997, Mr. Schmertzler held various management positions with Morgan Stanley and its affiliates, including President of Morgan Stanley Leveraged Capital Funds and head of Morgan Stanley's biotechnology pharmaceuticals group, and was Managing Director and Chief Financial Officer of Lehman Brothers Kuhn Loeb, an investment banking firm. Mr. Schmertzler is currently a director of Lehman Commercial Paper Incorporated, a liquidating post-bankruptcy subsidiary of Lehman Brothers Holdings, Incorporated. From 2008 to 2012, Mr. Schmertzler served as Chief Executive Officer and a director of Kolltan Pharmaceuticals, Inc., a Yale University biotechnology spin-out. Mr. Schmertzler previously served as a director of Cytokinetics, Incorporated, a public biopharmaceutical company and Idenix Pharmaceuticals, Inc. Since 1998, he has been an Adjunct Professor and Lecturer at Yale University. Mr. Schmertzler received a B.A. from Yale College in Molecular Biophysics and Biochemistry, History and City Planning and an M.B.A. from the Harvard Business School. We believe that Mr. Schmertzler is qualified to serve on our Board due to his extensive experience as an investment banking and financial professional, his extensive personal knowledge of our industry and his many years of service as one of our directors.

Defendant Aldrich

38. Defendant Richard Aldrich ("Aldrich") served as a Company director from March 2013 to June 2015. According to the 2015 Proxy Statement, as of April 20, 2015, Defendant Aldrich beneficially owned 23,333 shares of the Company's common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, Aldrich owned over $1.6 million worth of PTC stock.

39. For the fiscal year ended December 31, 2015, Defendant Aldrich received $377,603 worth of compensation from the Company. This included $17,802 in fees earned or cash paid and $359,801 option awards.

40. The Company's Schedule 14A filed with the SEC on April 28, 2014 (the "2014 Proxy Statement") stated the following about Defendant Aldrich:

> Mr. Aldrich has served as a member of our Board since March 2013. Mr. Aldrich has served as a partner of Longwood Fund, LP, a venture capital investment fund, since 2010. He founded RA Capital Management LLC, a hedge fund, in 2001 and served as a managing member from 2004 to 2008 and as a co-founding member from 2008 until 2011. He co-founded Sirtris Pharmaceuticals, Inc., which was acquired by GlaxoSmithKline plc in 2008, and served on its board of directors from 2004 to 2008; co-founded Concert Pharmaceuticals, Inc. and has served as Chairman of its board of directors since 2006; co-founded Alnara Pharmaceuticals, Inc., which was acquired by Eli Lilly in 2010, and served on its board of directors from 2008 to 2010; and co-founded OvaScience, Inc., a publicly traded life sciences company, and has served on its board of directors since 2011 and as Chairman of the board since 2012. Mr. Aldrich also joined Vertex Pharmaceuticals, Inc., a publicly traded biotechnology company, at its founding in 1989 and served as Senior Vice President and Chief Business Officer until 2001. Mr. Aldrich has also served on the board of directors of Verastem, Inc., a publicly traded biopharmaceutical company, since August 2010. Mr. Aldrich received a B.S. from Boston College and an M.B.A from the Amos Tuck School at Dartmouth College. We believe that Mr. Aldrich is qualified to serve on our Board because of his experience in the life sciences industry and as an entrepreneur and venture capital investor and his service on the boards of directors of other life sciences companies.

41. During the period of time when the Company materially misstated information to keep the stock price inflated, and before the scheme was exposed, Defendant Aldrich made the following sale of Company stock (and made no purchases of Company stock). On June 8, 2015, Defendant Aldrich sold 7,000 shares of Company stock for $55.85 per share. Thus, before the fraud was exposed, he sold 7,000 Company shares on inside information, for which he received $390,950. His insider sale, made with knowledge of material non-public information before the material misstatements and omissions were exposed, demonstrates his motive in facilitating and participating in the fraud.

Defendant Jacobson

42. Defendant Allan Jacobson ("Jacobson") has served as a Company director since the Company's inception in 1998, and is a co-founder of the Company. According to the 2015 Proxy Statement, as of April 20, 2015, Defendant Jacobson beneficially owned 64,430 shares of the Company's common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, Jacobson owned over $4.4 million worth of PTC stock.

43. For the fiscal year ended December 31, 2015, Defendant Jacobson received $487,801 worth of compensation from the Company. This included $38,000 in fees earned or cash paid, $359,801 in option awards, and $90,000 in all other compensation.

44. The Company's 2015 Proxy Statement stated the following about Defendant Jacobson:

> Dr. Jacobson is a co-founder of our company and has served as a member of our Board since our inception in 1998, and previously served as Chairman of our Board from 1998 to 2004. Since 2000, Dr. Jacobson has served as Chairman of our scientific advisory board. Since 1994, Dr. Jacobson has been the Chairman of the Department of Microbiology and Physiological Systems at the University of Massachusetts Medical School. In 1982, Dr. Jacobson co-founded Applied bioTechnology, Inc., a biotechnology company, and served as its chairman until its sale in 1991. From 1987 to 1990, Dr. Jacobson served as special limited partner at Euclid Partners, a venture capital firm. Dr. Jacobson received a Ph.D. from Brandeis University in 1971, has authored over 100 publications in the field of post-transcriptional control processes and is an elected member of the American Academy of Microbiology. We believe that Dr. Jacobson is qualified to serve on our Board because of his service as one of our directors since our inception, his knowledge of our company and his extensive experience as a founder and leader of new businesses in the life science industry.

45. During the period of time when the Company materially misstated information to keep the stock price inflated, and before the scheme was exposed, Defendant Jacobson made the following sales of Company stock (and made no purchases of Company stock). On March 18, 2015, Defendant Jacobson sold 16,616 shares of Company stock for $76.21 per share. On June

18, 2015, Defendant Jacobson sold 5,000 shares of Company stock for $50.13. Thus, before the fraud was exposed, he sold 21,616 Company shares on inside information, for which he received over $1.5 million. His insider sales, made with knowledge of material non-public information before the material misstatements and omissions were exposed, demonstrate his motive in facilitating and participating in the fraud.

Defendant Koppel

46. Defendant Adam Koppel ("Koppel") served as a Company director from March 2013 to June 2017. According to the 2015 Proxy Statement, as of April 20, 2015, Defendant Koppel beneficially owned 23,333 shares of the Company's common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, Koppel owned over $1.6 million worth of PTC stock.

47. For the fiscal year ended December 31, 2015, Defendant Koppel received $403,801 worth of compensation from the Company. This included $44,000 in fees earned or cash paid and $359,801 in option awards.

48. The Company's 2015 Proxy Statement stated the following about Defendant Koppel:

> Dr. Koppel has served as a member of our Board since March 2013. Since May 2014, Dr. Koppel has served as Senior Vice President and Chief Strategy Officer of Biogen Idec Inc., a biotechnology company. From 2003 to May 2014, Dr. Koppel served as a Managing Director of Brookside Capital, LLC, the public equity affiliate of Bain Capital. Prior to joining Brookside Capital, LLC, he was an Associate Principal with McKinsey & Company where he consulted companies in the pharmaceutical and biotechnology industries. Dr. Koppel received an M.D. and Ph.D. from the University of Pennsylvania School of Medicine, an M.B.A. from the Wharton School of the University of Pennsylvania and a B.A. from Harvard University. Since 2014, Dr. Koppel has served on the board of directors of Trevana, Inc. We believe that Dr. Koppel is qualified to serve on our Board due to his extensive experience as an investment banking and financial professional and his extensive knowledge of the pharmaceutical and biotechnology industries.

Defendant Kranda

49. Defendant Michael Kranda ("Kranda") served as a Company director from December 2003 to June 2015. According to the 2015 Proxy Statement, as of April 20, 2015, Defendant Kranda beneficially owned 25,937 shares of the Company's common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, Kranda owned over $1.8 million worth of PTC stock.

50. For the fiscal year ended December 31, 2015, Defendant Kranda received $1,006,560 worth of compensation from the Company. This included $29,000 in fees earned or cash paid, $948,560 in option awards, and $29,000 in all other compensation.

51. The Company's 2014 Proxy Statement stated the following about Defendant Kranda:

> Mr. Kranda has served as a member of our Board since December 2003. Since September 2006, Mr. Kranda has served as a consultant to Vulcan Capital, the private investment group of Vulcan Inc., and Mr. Kranda served as Managing Director of biotechnology venture investments at Vulcan Capital from September 2003 to September 2006. From July 1996 to July 2002, Mr. Kranda served as Chief Executive Officer at Oxford GlycoSciences, a biotechnology company. Prior to joining Oxford GlycoSciences, Mr. Kranda was President and Chief Operating Officer at Immunex Corporation (now Amgen), a biopharmaceutical company. Mr. Kranda currently serves as Chief Executive Officer and a director of BEAT BioTherapeutics Corporation, a gene therapy company. Mr. Kranda received a B.A. and an M.B.A from the University of Washington School of Business. We believe that Mr. Kranda is qualified to serve on our Board because of his many years of service as one of our directors, his extensive experience in the life sciences industry and his service on the boards of directors of other life sciences companies.

52. During the period of time when the Company materially misstated information to keep the stock price inflated, and before the scheme was exposed, Defendant Kranda made the following sale of Company stock (and made no purchases of Company stock). On March 16, 2015, Defendant Kranda sold 2,500 shares of Company stock for $72.93 per share. Thus, before the fraud was exposed, he sold 2,500 Company shares on inside information, for which he received

$182,325. His insider sale, made with knowledge of material non-public information before the material misstatements and omissions were exposed, demonstrates his motive in facilitating and participating in the fraud.

Defendant McDonough

53. Defendant C. Geoffrey McDonough ("McDonough") served as a Company director from November 2012 until his resignation on September 18, 2017. According to the 2015 Proxy Statement, as of April 20, 2015, Defendant McDonough beneficially owned 30,000 shares of the Company's common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, McDonough owned over $2 million worth of PTC stock.

54. For the fiscal year ended December 31, 2015, Defendant McDonough received $394,801 worth of compensation from the Company. This included $35,000 in fees earned or cash paid and $359,801 in option awards.

55. The Company's 2015 Proxy Statement stated the following about Defendant McDonough:

> Dr. McDonough has served as a member of our Board since November 2012. Since August 2011, Dr. McDonough has served as President and Chief Executive Officer of Swedish Orphan Biovitrum AB (Sobi), a Swedish pharmaceutical company. Prior to joining Sobi, Dr. McDonough held several senior leadership positions at Genzyme Corporation, a biotechnology company, from 2002 to June 2011, including Senior Vice President and General Manager, Personalized Genetic Health, Senior Vice President, Lysosomal Storage Disease Therapeutics and most recently, as President of Europe, Middle East and Africa. Prior to joining Genzyme, Dr. McDonough co-founded and served as President of Catalyst Medical Solutions, a developer of software for hospital management, and was a practicing internist and pediatrician. Dr. McDonough received a B.A. and a B.Sc. from the University of North Carolina at Chapel Hill and an M.D. from Harvard Medical School. We believe that Dr. McDonough is qualified to serve on our Board because of his extensive executive leadership experience and knowledge of our industry.

Defendant Renaud

56. Defendant Ronald C. Renaud, Jr. ("Renaud") served as a Company director from June 2014 to June 2017. According to the 2015 Proxy Statement, as of April 20, 2015, Defendant Renaud beneficially owned 6,666 shares of the Company's common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, Renaud owned over $463,020 worth of PTC stock.

57. For the fiscal year ended December 31, 2015, Defendant Renaud received $412,551 worth of compensation from the Company. This included $52,750 in fees earned or cash paid and $359,801 in option awards.

58. The Company's 2015 Proxy Statement stated the following about Defendant Renaud:

> Mr. Renaud has served as a member of our Board since June 2014. Since December 2014 he has served as the Chief Executive Officer of RaNA Therapeutics, Inc., a biotechnology company. Previously Mr. Renaud served as the president and chief executive officer of Idenix Pharmaceuticals, Inc., a public biopharmaceutical company, from October 2010 until its acquisition by Merck in August 2014. Mr. Renaud served as Idenix's chief financial officer from June 2007 to October 2010 and chief business officer from June 2010 to October 2010. Prior to joining Idenix, Mr. Renaud served as senior vice president and chief financial officer of Keryx Biopharmaceuticals, Inc., a biopharmaceutical company, from February 2006 to May 2007. He was a senior research analyst and global sector coordinator for JP Morgan Securities from May 2004 until February 2006, where he was responsible for the biotechnology equity research effort, covering all ranges of capitalized biotechnology companies. He also spent more than five years at Amgen, where he held positions in clinical research, investor relations and finance. Mr. Renaud holds a B.A. from St. Anselm College and an M.B.A. from the Marshall School of Business at the University of Southern California. Mr. Renaud serves on the boards of Chimerix, Inc. and Akebia Therapeutics since 2014. We believe that Mr. Renaud is qualified to serve on our Board because of his leadership and finance experience at public and private biotechnology companies, his investment banking background and his deep knowledge of the life sciences industry.

Defendant Southwell

59. Defendant David P. Southwell ("Southwell") has served as a Company director since 2005. He also serves as Chair of the Compensation Committee and as a member of the Audit Committee. According to the 2015 Proxy Statement, as of April 20, 2015, Defendant Southwell beneficially owned 39,318 shares of the Company's common stock. Given that the price per share of the Company's common stock at the close of trading on April 20, 2015 was $69.46, Southwell owned over $2.7 million worth of PTC stock.

60. For the fiscal year ended December 31, 2015, Defendant Southwell received $417,801 worth of compensation from the Company. This included $58,000 in fees earned or cash paid and $359,801 in option awards.

61. The Company's 2015 Proxy Statement stated the following about Defendant Southwell:

> Mr. Southwell has served as a member of our Board since December 2005. He is currently the President and Chief Executive Officer, and a member of the board of directors, of Inotek Pharmaceuticals, a biotechnology company. From March 2010 to September 2012, Mr. Southwell served as the Executive Vice President and Chief Financial Officer, and from 2008 to 2010 served as a member of the board of directors, of Human Genome Sciences, Inc., a biopharmaceutical company. Prior to joining Human Genome Sciences, he served as Executive Vice President and Chief Financial Officer of Sepracor, Inc., a research-based pharmaceutical company, from June 1994 to March 2008, and as Sepracor's Senior Vice President and Chief Financial Officer, from 1994 to 1995. From August 1988 until 1994, Mr. Southwell was associated with Lehman Brothers Inc., a securities firm, in various positions with the investment banking division. Since 2007, Mr. Southwell has served on the board of directors of THL Credit, Inc., a publicly traded business development company under the Investment Company Act of 1940 and from 2000 to 2010, he served on the board of directors of BioSphere Medical, Inc. Mr. Southwell received a B.A. from Rice University and an M.B.A. from the Tuck School of Business at Dartmouth College. We believe that Mr. Southwell is qualified to serve on our Board because of his extensive executive leadership experience and knowledge of our industry.

62. During the period of time when the Company materially misstated information to keep the stock price inflated, and before the scheme was exposed, Defendant Southwell made the

following sales of Company stock (and made no purchases of Company stock). On March 23, 2015, Defendant Southwell sold 2,317 shares of Company stock for $69.45 per share. On July 6, 2015, Defendant Southwell sold 23,604 shares of Company stock for $47.86 per share. Thus, before the fraud was exposed, he sold 25,921 Company shares on inside information, for which he received over $1.2 million. His insider sales, made with knowledge of material non-public information before the material misstatements and omissions were exposed, demonstrate his motive in facilitating and participating in the fraud.

Defendant Zeldis

63. Defendant Jerome Zeldis ("Zeldis") has served as a Company director since September 2012. He also serves as a member of the Nominating and Corporate Governance Committee.

64. For the fiscal year ended December 31, 2015, Defendant Zeldis received $394,801 worth of compensation from the Company. This included $35,000 in fees earned or cash paid and $359,801 in option awards.

65. The Company's 2015 Proxy Statement stated the following about Defendant Zeldis:

> Dr. Zeldis has served as a member of our Board since September 2012. Dr. Zeldis currently serves as the Chief Executive Officer of Celgene Global Health and the Chief Medical Officer of Celgene Corporation, a public biopharmaceutical company, where he has been employed since 1997. He previously served as Celgene's Senior Vice President of Clinical Research and Medical Affairs. Previously, Dr. Zeldis served as Assistant Professor of Medicine at Harvard Medical School, Associate Professor of Medicine at University of California, Davis, Clinical Associate Professor of Medicine at Cornell Medical School and Professor of Clinical Medicine at the Robert Wood Johnson Medical School. Dr. Zeldis received an A.B. and M.S. from Brown University and a M.Phil., M.D. and Ph.D. in Molecular Biophysics and Biochemistry (immunochemistry) from Yale University. Dr. Zeldis has served on the board of directors of Soligenix, Inc., a public biopharmaceutical company, since June 2011, on the board of directors of Alliqua, Inc., a public biomedical company, since May 2012, and founded and

> serves as the chairman of Trek Therapeutics, a biomedical company, since November 2014. We believe that Dr. Zeldis is qualified to serve on our Board because of his extensive executive leadership experience and knowledge of our industry.

Defendant Steele

66. Defendant Glenn D. Steele, Jr. ("Steele") has served as a Company director since June 2015. He also serves as a member of the Compensation Committee.

67. For the fiscal year ended December 31, 2015, Defendant Steele received $680,632 worth of compensation from the Company. This included $18,942 in fees earned or cash paid and $661,690 in option awards.

68. The Company's 2015 Proxy Statement stated the following about Defendant Steele:

> Dr. Steele has served as President and Chief Executive Officer of Geisinger Health System, an integrated health services organization in central and northeastern Pennsylvania, since March 2001. Dr. Steele previously served as the dean of the Biological Sciences Division and the Pritzker School of Medicine and vice president for medical affairs at the University of Chicago, as well as the Richard T. Crane Professor in the Department of Surgery. Prior to that, he was the William V. McDermott Professor of Surgery at Harvard Medical School, president and chief executive officer of Deaconess Professional Practice Group, Boston, MA, and chairman of the department of surgery at New England Deaconess Hospital (Boston, MA). Dr. Steele serves on the boards of director of several public companies, including, CEPHEID, Weis Markets Inc., and Wellcare Health Plans Inc. In addition, Dr. Steele serves on the governing body of several private organizations, including Bucknell University, xG Health Solutions, and Geisinger Health System. We believe that Dr. Steele is qualified to serve on our Board because of his leadership and business experience, extensive experience in the health care industry, and his service on the boards of directors of other public companies.

FIDUCIARY DUTIES OF THE INDIVIDUAL DEFENDANTS

69. By reason of their positions as officers, directors, and/or fiduciaries of PTC and because of their ability to control the business and corporate affairs of PTC, the Individual Defendants owed PTC and its shareholders fiduciary obligations of trust, loyalty, good faith, and due care, and were and are required to use their utmost ability to control and manage PTC in a fair,

just, honest, and equitable manner. The Individual Defendants were and are required to act in furtherance of the best interests of PTC and its shareholders so as to benefit all shareholders equally.

70. Each director and officer of the Company owes to PTC and its shareholders the fiduciary duty to exercise good faith and diligence in the administration of the Company and in the use and preservation of its property and assets and the highest obligations of fair dealing.

71. The Individual Defendants, because of their positions of control and authority as directors and/or officers of PTC, were able to and did, directly and/or indirectly, exercise control over the wrongful acts complained of herein.

72. To discharge their duties, the officers and directors of PTC were required to exercise reasonable and prudent supervision over the management, policies, controls, and operations of the Company.

73. Each Individual Defendant, by virtue of his or her position as a director and/or officer, owed to the Company and to its shareholders the highest fiduciary duties of loyalty, good faith, and the exercise of due care and diligence in the management and administration of the affairs of the Company, as well as in the use and preservation of its property and assets. The conduct of the Individual Defendants complained of herein involves a knowing and culpable violation of their obligations as directors and officers of PTC, the absence of good faith on their part, or a reckless disregard for their duties to the Company and its shareholders that the Individual Defendants were aware or should have been aware posed a risk of serious injury to the Company. The conduct of the Individual Defendants who were also officers and directors of the Company has been ratified by the remaining Individual Defendants who collectively comprised PTC's Board at all relevant times.

74. As senior executive officers and directors of a publicly-traded company whose common stock was registered with the SEC pursuant to the Exchange Act and traded on the NASDAQ, the Individual Defendants had a duty to prevent and not to effect the dissemination of inaccurate and untruthful information with respect to the Company's financial condition, performance, growth, operations, financial statements, business, products, management, earnings, internal controls, and present and future business prospects, including the dissemination of false information regarding the Company's business, prospects, and operations, and had a duty to cause the Company to disclose in its regulatory filings with the SEC all those facts described in this Complaint that it failed to disclose, so that the market price of the Company's common stock would be based upon truthful and accurate information.

75. To discharge their duties, the officers and directors of PTC were required to exercise reasonable and prudent supervision over the management, policies, practices, and internal controls of the Company. By virtue of such duties, the officers and directors of PTC were required to, among other things:

(a) ensure that the Company was operated in a diligent, honest, and prudent manner in accordance with the laws and regulations of Delaware, New Jersey, and the United States, and pursuant to PTC's own Code of Business Conduct and Ethics;

(b) conduct the affairs of the Company in an efficient, business-like manner so as to make it possible to provide the highest quality performance of its business, to avoid wasting the Company's assets, and to maximize the value of the Company's stock;

(c) remain informed as to how PTC conducted its operations, and, upon receipt of notice or information of imprudent or unsound conditions or practices, to make reasonable inquiry in connection therewith, and to take steps to correct such conditions or practices;

(d) establish and maintain systematic and accurate records and reports of the business and internal affairs of PTC and procedures for the reporting of the business and internal affairs to the Board and to periodically investigate, or cause independent investigation to be made of, said reports and records;

(e) maintain and implement an adequate and functioning system of internal legal, financial, and management controls, such that PTC's operations would comply with all applicable laws and PTC's financial statements and regulatory filings filed with the SEC and disseminated to the public and the Company's shareholders would be accurate;

(f) exercise reasonable control and supervision over the public statements made by the Company's officers and employees and any other reports or information that the Company was required by law to disseminate;

(g) refrain from unduly benefiting themselves and other Company insiders at the expense of the Company; and

(h) examine and evaluate any reports of examinations, audits, or other financial information concerning the financial affairs of the Company and to make full and accurate disclosure of all material facts concerning, *inter alia*, each of the subjects and duties set forth above.

76. Each of the Individual Defendants further owed to PTC and the shareholders the duty of loyalty requiring that each favor PTC's interest and that of its shareholders over their own while conducting the affairs of the Company and refrain from using their position, influence or knowledge of the affairs of the Company to gain personal advantage.

77. At all times relevant hereto, the Individual Defendants were the agents of each other and of PTC and were at all times acting within the course and scope of such agency.

78. Because of their advisory, executive, managerial, and directorial positions with PTC, each of the Individual Defendants had access to adverse, non-public information about the Company.

79. The Individual Defendants, because of their positions of control and authority, were able to and did, directly or indirectly, exercise control over the wrongful acts complained of herein, as well as the contents of the various public statements issued by PTC.

CONSPIRACY, AIDING AND ABETTING, AND CONCERTED ACTION

80. In committing the wrongful acts alleged herein, the Individual Defendants have pursued, or joined in the pursuit of, a common course of conduct, and have acted in concert with and conspired with one another in furtherance of their wrongdoing. The Individual Defendants caused the Company to conceal the true facts as alleged herein. The Individual Defendants further aided and abetted and/or assisted each other in breaching their respective duties.

81. The purpose and effect of the conspiracy, common enterprise, and/or common course of conduct was, among other things, to: (i) facilitate and disguise the Individual Defendants' violations of law, including breaches of fiduciary duty, unjust enrichment, waste of corporate assets, gross mismanagement, abuse of control, and violations of Section 14(a) of the Exchange Act; (ii) conceal adverse information concerning the Company's operations, financial condition, legal compliance, future business prospects and internal controls; and (iii) to artificially inflate the Company's stock price while six of the Individual Defendants engaged in insider sales.

82. The Individual Defendants accomplished their conspiracy, common enterprise, and/or common course of conduct by causing the Company purposefully or recklessly to conceal material facts, fail to correct such misrepresentations, and violate applicable laws. In furtherance of this plan, conspiracy, and course of conduct, the Individual Defendants collectively and individually took the actions set forth herein. Because the actions described herein occurred under

the authority of the Board, each of the Individual Defendants who is a director of PTC was a direct, necessary, and substantial participant in the conspiracy, common enterprise, and/or common course of conduct complained of herein.

83. Each of the Individual Defendants aided and abetted and rendered substantial assistance in the wrongs complained of herein. In taking such actions to substantially assist the commission of the wrongdoing complained of herein, each of the Individual Defendants acted with actual or constructive knowledge of the primary wrongdoing, either took direct part in, or substantially assisted in the accomplishment of that wrongdoing, and was or should have been aware of his or her overall contribution to and furtherance of the wrongdoing.

84. At all times relevant hereto, each of the Individual Defendants was the agent of each of the other Individual Defendants and of PTC, and was at all times acting within the course and scope of such agency.

PTC'S CODE OF CONDUCT

85. Pursuant to the Company's Code of Business Conduct and Ethics (the "Code of Conduct"), the Code of Conduct applies to all of the Company's officers, directors, and employees, and each of them is expected to read the Code of Conduct and work with each other to ensure that it is followed.

86. The Code of Conduct provides, as to "Conflicts of Interest," that:

> A conflict of interest occurs when you have a competing interest that may interfere with your ability to make an objective decision, when your personal interest interferes, or appears to interfere, with the interests of the Company. Each of us is expected to use good judgment and avoid situations that can lead to even the appearance of a conflict. A conflict of interest can arise whenever you, as an officer, director or Employee, take action or have an interest that prevents you from performing your Company duties and responsibilities honestly, objectively and effectively.

> Conflicts of interest may be actual or just a matter of perception. Since these situations are not always clear-cut, you need to fully disclose them to your manager, Human Resources, Legal or Compliance so that we can properly manage them.

87. The Code of Conduct provides, as to "Compliance with Laws, Rules and Regulations," that:[2]

> All PTC Employees must comply with all the laws, rules, and regulations of the United States and other relevant countries, as well as the states, counties, cities and other jurisdictions, applicable to the Company and its business. Many of the laws that apply to our business carry severe penalties for violations. The Company can be subjected to significant monetary fines and other criminal or civil sanctions. In addition, violations may results in sanctions against individual employees, including substantial fines and prison sentences in some cases. Allegations that the Company has violated the law may result in significant damages to the Company's reputation and its relationships with customers and other stakeholders. We all share an obligation to help ensure that the Company and its representatives comply with all applicable laws.

88. The Code of Conduct provides, as to "Insider Trading," that:

> We will not use information of PTC or information from our business partners for personal benefit. Employees, officers and directors who have material non-public information about the Company or other companies, including our suppliers and customers, as a result of their relationship with the Company, are prohibited by law and Company policy from trading in securities of the Company or such other companies, as well as from communicating such information to others who might trade on the basis of that information. To help ensure that you do not engage in prohibited insider trading and avoid even the appearance of an improper transaction, the Company has adopted an Insider Trading Policy, which is available from the Human Resources Department, Legal Department or on the Company's Intranet.
>
> If you are uncertain about the constraints on your purchase or sale of any Company securities or the securities of any other company that you are familiar with by virtue of your relationship with the Company, you should consult with Chief Legal Officer before making any such purchase or sale.
>
> Third parties may ask you for information concerning the Company. Employees, officers and directors (other than the Company's authorized spokespersons) must not discuss internal Company matters with, or disseminate internal Company information to, anyone outside the Company, except as required in the performance of their Company duties and after an appropriate confidentiality agreement is in

[2] The Code of Conduct defines "PTC Employees" as "all officers, directors, and employees of PTC and all of its subsidiaries and affiliates."

> place. This prohibition applies particularly to inquiries concerning the Company from the media, market professionals (such as securities analysts, institutional investors, investment advisers, brokers and dealers) and security holders. All responses to inquiries on behalf of the Company must be made only by the Company's authorized spokespersons. If you receive any inquiries of this nature, you must decline to comment and refer the inquirer to your supervisor or one of the Company's authorized spokespersons. The Company's policies with respect to public disclosure of internal matters are described more fully in the Company's Disclosure Policy, which is available from the Human Resources Department or on the Company's Intranet.
>
> You also must abide by any lawful obligations that you have to your former employer. These obligations may include restrictions on the use and disclosure of confidential information, restrictions on the solicitation of former colleagues to work at the Company and non-competition obligations.

89. The Code of Conduct provides, as to "Protection and Proper Use of Company Assets," that:

> Proper protection and use of Company assets and assets entrusted to it by others, including proprietary information, is a fundamental responsibility of each Company Employee. Employees must comply with security programs to safeguard such assets against unauthorized use or removal, as well as against loss by criminal act or breach of trust. Outlined below are specific provisions for the protection of Company's property.
>
> We are each personally responsible for protecting Company assets and using them with care. Company assets include funds, facilities, equipment, information systems, intellectual property and confidential information.
>
> Personal use of Company assets is discouraged. All information that is sent or received through our computer or phone systems is part of official Company records, and we can be legally required to show those records. Therefore, make sure that business information you process is accurate, appropriate, ethical, and legal.
>
> Employees, officers and directors should seek to protect the Company's assets. Theft, carelessness and waste have a direct impact on the Company's financial performance. Employees, officers and directors must use the Company's assets and services solely for legitimate business purposes of the Company and not for any personal benefit or the personal benefit of anyone else.
>
> Employees, officers and directors must advance the Company's legitimate interests when the opportunity to do so arises. You must not take for yourself personal opportunities that are discovered through your position with the Company or the use of property or information of the Company.

90. The Code of Conduct provides, as to "Accuracy of Books and Records and Public Reports," that:

> Employees, officers and directors must honestly and accurately report all business transactions in all applicable reporting media maintained by the Company. You are responsible for the accuracy of your records and reports. Accurate information is essential to the Company's ability to meet legal and regulatory obligations.
>
> All Company books, records and accounts shall be maintained in accordance with all applicable regulations and standards and accurately reflect the true nature of the transactions they record. The financial statements of the Company shall conform to generally accepted accounting rules and the Company's accounting policies. No undisclosed or unrecorded account or fund shall be established for any purpose. No false or misleading entries shall be made in the Company's books or records for any reason, and no disbursement of corporate funds or other corporate property shall be made without adequate supporting documentation.

91. The Code of Conduct provides, as to "Accuracy of Public Reports," that:

> It is the policy of the Company to provide full, fair, accurate, timely and understandable disclosure in reports and documents filed with, or submitted to, the Securities and Exchange Commission and in other public communications.

92. The Code of Conduct provides, as to "Concerns Regarding Accounting or Auditing Matters," that:

> Employees with concerns regarding questionable accounting or auditing matters or complaints regarding accounting, internal accounting controls or auditing matters should utilize the reporting methodology outlined in the first section of this Code. All such concerns and complaints will be forwarded to the Audit Committee of the Board of Directors, unless they are determined to be without merit by the Chief Legal Officer and Principal Financial Officer of the Company.
>
> A record of all complaints and concerns received will be provided to the Audit Committee each fiscal quarter, regardless of whether the complaints were determined to be without merit pursuant to the preceding paragraph. Any such concerns or complaints may also be communicated, confidentially and, if you desire, anonymously, directly to the Chairman of the Audit Committee of the Board of Directors. The Audit Committee will evaluate the merits of any concerns or complaints received by it and authorize such follow-up actions, if any, as it deems necessary or appropriate to address the substance of the concern or complaint.

93. The Code of Conduct provides, as to "Dealings with Independent Auditors," that:

> No employee, officer or director shall, directly or indirectly, make or cause to be made a materially false or misleading statement to an accountant in connection with (or omit to state, or cause another person to omit to state, any material fact necessary in order to make statements made, in light of the circumstances under which such statements were made, not misleading to, an accountant in connection with) any audit, review or examination of the Company's financial statements or the preparation or filing of any document or report with the SEC. No employee, officer or director shall, directly or indirectly, take any action to coerce, manipulate, mislead or fraudulently influence any independent public or certified public accountant engaged in the performance of an audit or review of the Company's financial statement.

94. The Code of Conduct provides, as to "Compliance," that:

> Every Employee of the Company is responsible for conducting him or herself in a manner consistent with this Code.
>
> * * *
>
> Failure to follow this Code may subject an Employee to disciplinary action, up to and including termination. The assignment of any Agent who fails to follow this Code may be ended. Any Employee or Agent who becomes aware of an actual or potential violation of this Code or any PTC policy must promptly report it to his or her manager, and/or one of the following PTC departments: Compliance, Legal, Human Resources, or the PTC Confidential and Anonymous Hotline . . . .

95. In violation of the Code of Conduct, the Individual Defendants conducted little, if any, oversight of the Company's engagement in the Individual Defendants' scheme to issue materially false and misleading statements to the public and to facilitate and disguise the Individual Defendants' violations of law, including breaches of fiduciary duty, gross mismanagement, abuse of control, waste of corporate assets, unjust enrichment, and violations of Section 14(a) of the Exchange Act. Moreover, in violation of the Code of Conduct, the Individual Defendants failed to comply with laws and regulations, maintain the accuracy of Company records and reports, and uphold the employee and director responsibilities related thereto.

INDIVIDUAL DEFENDANTS' MISCONDUCT

Background

PTC and Translarna

96. PTC, founded in 1998, is biopharmaceutical company that focuses on the discovery, development, and commercialization of orally administered therapies to treat underserved diseases and disorders, utilizing post-transcriptional control, a process that treats deficiencies caused by genetic mutations that prevent proper gene expression. The Company's efforts are primarily geared toward treatments for orphan and ultra-orphan disorders. Orphan designation is given to drugs for the treatment of diseases that affect fewer than 200,000 people in the U.S., while ultra-orphan generally refers to a prevalence of 1-in-2,000 to 1-in-50,000 in the overall population. Since orphan and ultra-orphan disorders are so rare, the profitability of commercialization of treatments for such disorders is generally dependent on the treatments commanding some of the highest prices in the pharmaceutical industry.

97. Translarna, which PTC began developing in 2003, seeks to use post-transcriptional control to target and treat nonsense mutation, a specific genetic mutation that prevents the production of full-length, functional proteins which can result in a variety of genetic disorders depending on the protein, such as cystic fibrosis and DMD. Translarna, theoretically, slows the progression of a disorder by allowing a cell to "read-through" the nonsense mutation to produce functional proteins. Translarna was the Company's most advanced product candidate by far during the Relevant Period, and was the first drug product that PTC would seek regulatory approval for in the U.S. and Europe. Despite the Company having other product candidates under development, they were all years away from commercialization, and thus Translarna was the Company's only avenue for generating any revenues in the foreseeable future. As confirmed by a securities analyst from Roth Capital Partners, "PTC is all about Translarna, the success or failure of Translarna is likely to define the Company over the next year or so . . . ."

98. Per the theory underlying post-transcriptional control, Translarna could be used to theoretically treat any disorder that is caused by a nonsense mutation, thus making Translarna potentially unique. During the Relevant Period, the Company was furthest along in commercializing Translarna for the treatment of nmDMD. Since a successful trial and NDA for Translarna for DMD would greatly influence the FDA's overall opinion of Translarna (for this indication and any others), the success of the first Translarna clinical trials and subsequent FDA approval process for DMD were crucial.

99. DMD, a specific type of muscular dystrophy, was the first disorder targeted by the Company for treatment with Translarna. DMD occurs in young males predominantly, who usually start exhibiting symptoms from as early as two years old. The young children with DMD face issues with walking and standing, with most patients unable to walk by adolescence, generally due to a slow weakening and eventual wasting of muscle tissue. The catastrophic muscle loss in those suffering from DMD primarily results from their body's inability to properly produce dystrophin, a protein that normally supports the structure of muscle cells. Muscle cells break down from normal muscle use without dystrophin, and as fat or other fibrous cells take the place of damaged muscle cells, muscle function declines. DMD patients also suffer from weakened respiratory function beginning around the age of 10, requiring the child to use a respirator. Eventually, DMD impacts the heart muscles, which leads to heart failure and death.

100. Throughout much of Translarna's development, the Company benefitted from the FDA's orphan drug designation since nmDMD affects only a small patient population. As a result of the Orphan Drug Act, the FDA's orphan drug designation provided the Company with numerous development incentives, such as market exclusivity and tax credits for qualified clinical testing. The Company also received a grant through the FDA's Orphan Products Clinical Trial Grants

Program due to its development of an orphan drug, which funds clinical trials that a company must run in order to gain FDA approval for the drug. Since a clinical trial usually costs $30-40 million to design and implement, this financial assistance was especially valuable to PTC. Despite the assistance given to developers of orphan disorder drugs as a result of the orphan drug designation, the FDA strictly notes that "[t]he granting of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval. Safety and effectiveness of a drug must be established through adequate and well-controlled studies."

101. The Company also obtained "fast track" designation for Translarna, which is an FDA program that facilitates the development and expedites the review of drugs intended to treat serious conditions and unmet medical needs. The Company's fast track designation entitled PTC to take advantage of several benefits, such as more frequent meetings and communications with the FDA regarding the development of the drug and trials, and rolling review, which allows a company to submit a NDA for review as data analyses and other portions of the application come in, instead of having to wait for the entire NDA to be finished. Despite this designation, the onus was on PTC to take advantage of the benefits. As noted by the FDA:

> Once a drug receives Fast Track designation, early and frequent communication between the FDA and a drug company is encouraged throughout the entire drug development and review process. The frequency of communication assures that questions and issues are resolved quickly, often leading to earlier drug approval and access by patients.

As evidenced by the ultimate rejection of the Company's 2016 NDA for Translarna for nmDMD by the FDA, PTC was not in close communication with the FDA and failed to take advantage of the benefits resulting from the fast track and orphan drug designations it received.

The NDA Process

102. PTC was required to engage in the standard process of FDA drug approval and file an NDA prior to selling and marketing a treatment in the U.S. All drugs that are currently marketed in the U.S. were the subject of an NDA that was ultimately approved. The NDA must show "substantial evidence" that the drug is safe and effective at treating the condition it purportedly treats. Moreover, a properly submitted NDA will provide the FDA with all pertinent information regarding the drug, including statistical analyses and data sufficient to determine whether the benefits of the drug outweigh its risks, the drug is safe and provides the benefits it purports to, the proposed labeling of the drug is appropriate and what it should contain, and that the controls used to maintain the drug's quality and the methods used in manufacturing the drug are adequate to preserve the drug's identity, strength, quality, and purity. Each of these is independently critical to the FDA's ultimate approval decision, with safety and efficacy being particularly important in the FDA's assessment.

103. Drug developers usually subject a drug candidate to a series of clinical trials which are designed to accumulate the requisite data to submit a successful NDA, and are engaged in to meet the above referenced standards. Phase 1 clinical trials generally evaluate an investigational drug's safety and dosage tolerance, while Phase 2 clinical trials typically involve larger patient populations, evaluate dosage tolerance and appropriate dosage, identify possible short-term adverse effects and safety risks, and provide a preliminary evaluation of the efficacy of the drug for specific indications.

104. Phase 2 clinical trials can be conducted in divided sub-phases which are designated as Phase 2a and Phase 2b. While Phase 2a trials are usually smaller and provide additional introductory information on a drug's efficacy and safety, they are insufficient to independently support a NDA. Phase 2b trials, however, are typically larger, may be for longer periods of time,

and can support an NDA. In the Phase 2b trial for Translarna (the "Phase 2b Trial"), as the case in most Phase 2b trials, an investigational drug's efficacy is measured against a placebo.

105. Phase 3 clinical trials test for a drug's efficacy and safety in an expanded patient population, typically involve comparison with a placebo, and are intended to establish the overall risk-benefit profile of the drug product and provide an adequate basis of physician labeling of the drug.

106. Usually, the FDA requires two successful clinical trials to provide the FDA with "substantial evidence" that a drug is safe and effective. The FDA also requires that the trials be "adequate and well-controlled investigations," with the FDA emphasizing the plural in "investigations," indicating that most approvals of drugs are based on the results of two pivotal trials at least.

107. Per FDA procedures, a "submission" and a "filing" serve as two distinct events, where a company "submits" a NDA to the FDA, and after a preliminary review for facial sufficiency, the FDA "files" the application into a dossier, and the application remains there until regulators take further action for a complete substantive review. Accordingly, "filing" a submission is a decision by the FDA that the submission merits the resources required for complete substantive review. PTC submitted its NDA for Translarna for nmDMD through a rolling review process while its Phase 3 ACT DMD clinical trial was ongoing.

108. If the FDA receives an application, however, that is incomplete, improperly constructed, or otherwise facially inadequate, it issues an RTF letter (or "RTF") to the drug developer informing it that their application will not receive further agency attention until the problems with the application are corrected. As specifically noted by the FDA:

> [i]n general, a RTF is based on omissions of clearly necessary information . . . or omissions or inadequacies so severe as to render the application incomplete on its

> face and where the omissions or inadequacies are so obvious, at least once identified, and not a matter of interpretation or judgment about the meaning of the data submitted.

RTFs do not address issues related to the magnitude of clinical effect, nuances of study design, balancing or risks and benefits, or other complex issues and close judgments, as these issues are assessed after filing, which is when the FDA determines whether to approve an NDA.

109. There is no way to determine from publicly available information precisely how many NDA submissions are rejected via RTF prior to substantive FDA review, since the FDA does not release such information and also publicly traded companies are not obligated to disclose their receipt of any RTF unless they would experience it as a material event. All available resources, however, indicate that for applications for new molecular entities, the category that the Company's Translarna application falls in, RTFs are relatively rare. All available resources indicate that of the more than 200 applications submitted between January 2010 and February 2016, only 18 applications received RTFs—two of which concerned Translarna.

110. One reason for the relative unlikelihood of receiving an RTF is that modern drug development, particularly for orphan drugs, is premised on the availability of frequent communications between the FDA and drug developers and any issues or concerns that might be expressed by the FDA should be addressed prior to the completion of the submission.

Translarna's First Major Efficacy Trial

111. The Company's Phase 1 and Phase 2a trials for Translarna had been completed by May 2007, and PTC started designing the Phase 2b Trial—the first major trial intended to demonstrate that Translarna is effective in treating children with nmDMD. In February 2008, the Company began enrolling patients in the Phase 2b Trial, ultimately including 174 nmDMD patients between the ages of 5 and 20. At the time, it was the largest randomized, double-bling,

placebo-controlled DMD clinical trial in history. The Company received partial funding from federal funds in the form of a grant from the FDA Orphan Products Clinical Trials Grant Program, further evidencing that the FDA was more than available to assist the Company in approval of Translarna, and that the Company failed to take full advantage of the FDA's expertise and assistance.

112. The design of the clinical trial was intended to reveal a slower decline in a Translarna patient's ability to use their muscles, measured by the distance a patient could walk over six minutes. At week one of the trial—the baseline—the distance was to be measured and then measured again at week 48—the end. The findings for the patient population treated by Translarna were compared against the findings for DMD patients taking a placebo. It was the Company's belief that, if Translarna effectively slowed the degeneration of muscle, patients receiving Translarna would experience a smaller decline in their 6-minute walking distance over a 48-week period than would those taking the placebo.

113. Before the Phase 2b Trial was initiated, the Company pre-specified the criteria it would use to establish Translarna's effectiveness at treating nmDMD and set a "goalpost"—that Translarna would be deemed effective at treating nmDMD only if the distance walked by patients on the drug exceeded that of patients on the placebo by more than an average of 30 meters as of the end of the trial. Meeting this goalpost would represent a clinically meaningful benefit for the purposes of the trial and show that the ability of patients on Translarna to walk declined less. In order to rule out the probability of a false positive result, the Company also pre-specified that a benchmark p-value of 0.05 or less—meaning that there is only a 5% likelihood that the outcome is the result of chance alone—would have to be achieved by the results to be considered statistically significant and deemed demonstrative of the clinical effect of Translarna.

114. In December 2009, the Translarna Phase 2b Trial was completed and the Company released the preliminary results of the trial on March 3, 2010, with test results showing some slight improvements for the Translarna patients. However, the overall results did not meet the Company's self-specified criteria for clinically meaningful benefit and also were far from the required statistical significance. Thus, it was not demonstrated by the Phase 2b Trial that Tranlarna was effective at treating nmDMD.

115. The Company attributed the lack of statistical significance and disappointing results to "younger patients and patients with higher baseline 6-minute walk distances [that] are less likely to exhibit measurable declines in 6-minute walk distance over 48 weeks." PTC noted that these specific younger children that suffered from DMD were not progressed far enough in the disorder at the time of their initial assessments to experience a likely clinical effect during the course of the clinical trial.

116. Despite the poor results, PTC stated in its press releases, earnings calls, and SEC filings that the trial was a success and that the trial demonstrated Translarna's efficacy. PTC's statements and portrayed enthusiasm, however, was premised on improper post-hoc data analyses whereby the Company was purportedly able to show a statistically significant benefit for only a small sub-segment of the original patient population that the Company intended to treat in Translarna's Phase 2b Trial. FDA approval of a drug cannot be obtained through the use of such post-hoc analyses if it does not support the trial's primary endpoint. This is because they provide drug developers with the chance to manipulate the trial data's interpretation by specifically choosing only what most favorably demonstrates the effectiveness of the drug.

117. In March 2011, the Company nonetheless submitted a NDA based on the Translarna Phase 2b clinical trial's results (the "2011 NDA"), presumably with the belief that the

FDA would disregard the trial's failure to meet its overall criteria by which to measure the effectiveness of the drug. As part of their NDA, with the understanding that the FDA would view the trial's results as a failure, the Company additionally submitted post-hoc analyses focusing on an older, declining sub-segment of the trial patient population. The Company took the position that this sub-segment had shown a clinical benefit and further supported PTC's claims that the data from younger children in the trial whose DMD was less progressed was the cause of the Phase 2b Trial's negative results.

118. Unsurprisingly, the FDA sent the Company an RTF not long after (the "Phase 2b RTF"), noting that the NDA was facially deficient on the grounds that "the single placebo controlled Phase 2b clinical trial contained in the NDA did not achieve statistical significance in the pre-specified analysis." This was something that PTC had already known, and the Company nonetheless filed a formal dispute resolution request with the FDA in December 2011 regarding the Phase 2b RTF. The FDA reaffirmed the appropriateness of its decision in January 2012.

119. Following the notice received from the FDA that it would not hear the Company's appeal, the Company's executives in February 2012 took part in numerous meetings and discussions with the FDA regarding how to get approval for Translarna. The Company had discussions in these meetings with the very regulators who would be reviewing PTC's submission, which provided the opportunity to determine precisely what the FDA needed as "substantial evidence" of the efficacy of Translarna. The Company's executives additionally were provided with feedback regarding the deficiencies found by the FDA in the Phase 2b NDA. Thus, the Company was given the opportunity to receive feedback from the FDA, which would be the body to adjudicate the acceptability of their Phase 3 trial design, statistical analysis plan, and proposed endpoints.

120. The Company and Individual Defendants were therefore able to lower the likelihood of failure in designing the study and the statistical analysis plan by implementing the feedback and advice obtained from the FDA regarding the specific means by which the FDA would assess the drug's efficacy and by tailoring the design based on the results that were observed in the Phase 2b Trial. As confirmed by Defendant Peltz during an August 12, 2013 earnings call, "The design of the trial reflects the knowledge gained from our earlier study as well as the views expressed in discussions with the FDA . . . ." As discussed further herein, Defendants on numerous occasions disclosed that some of the meetings with the FDA concerned the Company's detailed statistical analysis plan that was submitted to the FDA for the Phase 2b trial.

121. The Phase 3 trial, or ACT DMD, was designed by the Company as a "confirmatory" study and intended to ratify the purported findings of efficacy from the Phase 2b Trial. As discussed herein, PTC would state this several times during the Relevant Period. As a result of the Phase 3 trial's refined enrollment criteria, the Phase 3 trial was attempting to confirm the positive results from one of the subgroups whose data in the Phase 2b Trial the Company analyzed post-hoc. Specifically, enrollment in ACT DMD, also known as "Study 020," was limited to DMD patients who could still walk and aged seven to 16 years old. These were the children that PTC predicted would demonstrate the greatest response Translarna based on the Phase 2b Trial's results, and the intention was to heavily focus on enrolling patients in a specific subgroup of DMD patients in the "decline phase," as termed by the Company.

122. The design of the trial was formulated based on the Phase 2b Trial, the results of which developed PTC's belief that patients who were able to walk less than 350 meters in six minutes were thought to be experiencing a less advanced form of DMD and therefore were more stable, but still were likely to show a significant decline over the course of 48 weeks. To further

bolster the study, the Company restricted the enrollment further by also eliminating boys in the advanced stages of DMD who were unable to walk.

123. The Company's restrictions on the ACT DMD's enrollment to these patients greatly increased the likelihood that PTC could provide sufficient evidence of the efficacy of Translarna in treating nmDMD. In tailoring this specific enrollment, the Company excluded many of its target customers that PTC would seek approval to market and sell Translarna to, specifically children under the age of seven with more stable DMD and those patients whose muscular dystrophy was too advanced for them to achieve the Company's desired results. PTC's strategy nevertheless provided the Company with the greatest odds of showing the drug's effectiveness in a small segment of the overall nmDMD patient population and was a strategy that the Company stated "wrung out the risk." PTC adopted this strategy despite its intention to seek approval of Translarna for the nmDMD population in entirety, including the members of that population who showed no demonstrated effect in clinical trials.

124. In Spring of 2015, the Company submitted its draft statistical analysis plan and trial design to the FDA, and the FDA discussed PTC's draft plan with the Company before providing final comments and signing off on it. It would later be revealed that the Defendants did not discuss with the FDA that they would use an unapproved post-hoc analysis as their "main analysis."

125. The design of the ACT DMD trial used the same benchmark goals as the Phase 2b Trial, and the primary goal of the trial would be the same goal as the Phase 2b Trial—which was a clinically meaningful and statistically significant change in 6-minute walk distance at 48 weeks as compared to week one. A clinically meaningful benefit, just as the Phase 2b Trial, was defined at the threshold of 30 meters and the level of statistical significance was, just as the Phase 2b Trial, the 0.05 benchmark. Per the Company's Form 10-Q filed with the SEC on November 9, 2015

after the release of the ACT DMD results, the statistical analysis plan also included numerous pre-specified subgroups, such as the key < 350 meter subgroup that PTC had designed the trial around. The Company noted this < 350 meter subgroup was "a key subgroup based on the knowledge that 350 meters represents a transition point for patients towards a more rapid decline in walking ability as supported by analysis from our Phase 2b study." The statistical analysis plan also included at least one other pre-specified subgroup that consisted of patients who walked between 300 and 400 meters during the baseline walking tests. This 300-400 meter subgroup was included by the Company "based on an increasing understanding of the sensitivity limitations of the six minute walk tests as an endpoint in 48-week studies." Before the Company released the results of the ACT DMD, PTC had not disclosed the 300-400 meter subgroup's existence to the public.

126. Moreover, a "meta-analysis" was included in the statistical analysis plan which combined the "efficacy results from the ACT DMD ITT population and Phase 2b ambulatory decline phase subgroup." The Company greatly increased its likelihood of being able to present a statistically significant result to the FDA by including this meta-analysis, resulting from the substantially larger patient population that data could be drawn from. Specifically, the included meta-analysis would combine and aggregate the results from the Phase 2b Trial that PTC described as positive with the ACT DMD trial's overall results, in order to attempt to show the efficacy of Translarna.

127. In October 2015, the Company announced the results of the ACT DMD Phase 3 study, although it did not disclose the full trial data. The study, as it had in its prior iteration, again failed to show a statistically significant result for the primary endpoint. In fact, the primary endpoint analysis demonstrated substantially worse results than those from the Phase 2b Trial. Also, the 6-minute walk distance for the overall population in the study was worse than the result

in the Phase 2b trial and significantly below the pre-specified threshold for evidencing a clinically meaningful benefit that had been set by PTC. Moreover, the key < 350 meter subgroup that PTC had designed the Phase 3 trial around, which also was the main reason PTC stated that the trial was "powered for success," failed to show statistical significance or a clinically meaningful benefit.

128. The Company, despite acknowledging that its study did not meet the required level of statistical significance for the key < 350 meter subgroup or for the primary endpoint for the overall population, touted positive trial results because they were able to show a statistically significant and clinically meaningful result for the small 300-400 meter subgroup. The Company stated that "pre-specified meta-analysis of [its] combined Phase 2b and ACT DMD results" supported these positive trial results. Despite Defendants' claims, however, the results were not clinically meaningful even if they were to be considered statistically significant. Thus, they still did not meet the trial's primary clinical endpoint chosen by the Company with the assistance of the FDA to indicate and define success.

129. The Company attempted to gain something out of the Phase 3 trial's results by reporting a second meta-analysis that hand-picked what the Defendants now knew was the best performing subgroup in the trial, and combined it with patients who fit that subgroup in the completed Phase 2b Trial. This retrospective meta-analysis, predictably, demonstrated a very high level of statistical significance and clinically meaningful benefit. Notably, the Company did not pre-specify this second meta-analysis prior to the beginning of the Phase 3 trial. This meant that it was designed specifically for the purpose of manufacturing a way to reshape the failed trail as a success and also to mislead the public intentionally as to the true effectiveness of Translarna, since the second meta-analysis was developed only after the trial results were known and the clinical

trial data was fully digested. PTC's emphases placed on subgroups and meta-analyses misled the public into having the impression that the Company was able to show in two pre-specified analyses that Translarna demonstrated a clinically meaningful result that was statistically significant to a very high degree.

130. The Phase 3 trial population should have led to stronger evidence of efficacy than the Phase 2b Trial since the Phase 3 trial population was designed around the subgroup that PTC stated experienced the biggest benefit from Translarna in Phase 2b. The trial, however, could only show that the overall ITT population that had been treated with Translarna experienced approximately half the benefit purportedly observed in the prior trial, with even worse statistical significance, despite the Phase 2b data purportedly demonstrating that the treatment population experienced some improvement over the placebo population that was not clinically meaningful and that even this sub-par improvement (29.7 meters) was not statistically significant.

131. PTC failed to address the fact that Phase 3 trial patients outside of the 300-400 meter subgroup, which constituted approximately 57% of the overall ITT population, saw no clinically meaningful or statistically significant benefit from treatment with Translarna, instead merely addressing the beneficial results in the 300-400 meter subgroup. Translarna had not been shown to work in patients outside of the 300-400 meter subgroup and showed even less effectiveness in the overall population than data gathered from the Phase 2b Trial. Thus, it is evident that Translarna's efficacy was not confirmed in the ACT DMD trial. In fact, the Phase 3 trial's results instead undermined aspects of the Phase 2b study, which the Company had used as the reason for stating that the Phase 3 trial was "powered for success." The Phase 3 trial, in addition to demonstrating notably worse results in the overall patient population, was not able to "confirm"

statistical significance or a clinically meaningful benefit in the subgroup that the entire study was predicated on, the < 350 meter subgroup.

132. Despite these failures, the Defendants, as discussed further herein, misleadingly and repeatedly reassured the public that the ACT DMD trial's results demonstrated efficacy of the type that would be necessary to gain FDA review and subsequent approval. Their false and misleading statements created a public impression that, despite the disappointing results from the Company's trials, the evidence gathered from them would be sufficient to file with the FDA an NDA that would be approved thereafter.

PTC's Submission of the ACT DMD Trial Data

133. After the release of the initial Phase 3 trial results, PTC announced in January 2016 that its second NDA submission (the "2016 NDA") was completed. The 2016 NDA was for full approval and used data from the Phase 3 and Phase 2b trials as support. It sought a broad-label approval, specifically seeking approval for the treatment of all nmDMD patients regardless of the extent the disease progressed.

134. In further support of the 2016 NDA, the Company depended on the supposedly favorable 300-400 meter subgroup findings and the pre-specified meta-analysis combining data from the Phase 3 and Phase 2b trials and which PTC stated "provide[d] substantial evidence of the effectiveness of Translarna . . . for the treatment of nmDMD." Purportedly, the inclusion of the meta-analyses by the Company would meet the FDA requirement that drug sponsors demonstrate "substantial evidence" that a drug is safe and effective, "based on the results of at least two pivotal trials," despite that neither of the trials were able to meet their primary endpoints. The public, generally unfamiliar with the regulatory threshold for demonstrating the efficacy of a drug and the required statistical analyses to prove it, would not be able to decipher the actual import of the data

from the trial, nor realize that the Defendants were misleadingly underrepresenting the elevated risks of FDA rejection known by them.

135. The Company moreover relied on a post-hoc statistical analysis of the ACT DMD trial's 300-400 meter subgroup to make the claim it demonstrated Translarna's effectiveness at treating nmDMD. By using this non-pre-specified analysis, PTC was able to eliminate data from nearly 60% of the study population, and was its greatest evidence supporting the notion that Translarna was actually effective. The Defendants were well aware that demonstrating efficacy in only a small sub-group of the study population would not be enough to get the FDA to accept the NDA and garner full approval, and that such manufactured results, while apparently impressive as a result of the Company's use of post-hoc analyses, would eventually be deemed improper by the FDA. As noted by Defendant Kovacs while discussing why the ACT DMD study was poised for success during the Wedbush PacGrow Healthcare Conference in August 2015, "clearly the drug should have benefit across the whole population."

PTC's NDA Not Accepted for Filing by the FDA

136. On February 22, 2016, the Company received the RTF Letter from the FDA, discussed in further detail herein, relaying to PTC that its NDA for Translarna was "not sufficiently complete to permit a substantive review" of the application. In discussion of the letter, Defendants stated "there were really two bases . . . that were outlined in the letter; the first of which was that both the Phase 2b and Phase 3 studies had failed and therefore did not demonstrate substantial evidence of effectiveness and then secondly that the application did not sufficiently describe the abuse potential of the drug."

137. On July 25, 2016, the Company announced via press release that it had appealed the RTF Letter, and on October 17, 2016, PTC announced that the appeal was denied.

False and Misleading Statements

November 6, 2014 Form 10-Q

138. On November 6, 2014, the Company filed a Form 10-Q with the SEC for the third fiscal quarter ended September 30, 2014 (the "3Q 2014 10-Q"), signed by Defendant Kovacs, announcing that PTC would initiate its submission of information for the 2016 NDA on a rolling basis. Defendant Kovacs noted the benefits of the rolling review of its NDA for Translarna for nmDMD, stating, in relevant part:

> We also plan to initiate a rolling new drug application, or NDA, with the U.S. Food and Drug Administration, or FDA, for Translarna as a treatment for nmDMD. We currently anticipate that we will commence the submission process before the end of 2014. We believe this process gives the FDA an opportunity to conduct a meaningful review of most of the segments of our NDA, ahead of reviewing our Phase 3 ACT DMD data. We expect that the submission of the ACT DMD data will complete our rolling NDA.

139. Attached to the 3Q 2014 10-Q were certifications pursuant to Rule 13a-14(a) and 15d-14(a) under the Exchange Act and the Sarbanes-Oxley Act of 2002 ("SOX") signed by Defendants Peltz and Kovacs attesting to the accuracy of the 3Q 2014 10-Q.

November 6, 2014 Earnings Call

140. On November 6, 2014, the Company held an earnings call to discussing its third quarter 2014 financial results and the status of the ACT DMD clinical trial. During the call, Defendant Peltz expressed positive expectations pertaining to the FDA's review of the 2016 NDA, specifically noting that the FDA would not only be reviewing the Translarna NDA, but, per the FDA, the Translarna NDA would be reviewed on an expedited basis. Defendant Peltz stated, in relevant part:

> Regarding regulatory approval of Translarna for DMD patients in the US, we've been in dialog with the FDA. We now intend to initiate a rolling NDA submission by the end of this year for the approval of Translarna for DMD in the US. This process gives the FDA an opportunity to conduct meaningful review of most of the segments of our NDA ahead of reviewing our Phase 3 ACT DMD data. We expect

> that the submission of this confirmatory Phase 3 data will complete our rolling NDA. Based on our discussions with the FDA, we expect that our rolling NDA will be rapidly reviewed for potential approval within the first half of 2016.

141. Defendant Peltz also discussed the commercial launch timeframe of Translarna, stating, in relevant part:

> I think that the way we're thinking about this is that, as I said, the discussions with the FDA that will get the various segments of the NDA in and then on completion of ACT DMD trial, the data will come out. We'll then expeditiously get it in and it's our hope and I think in our dialog with them, given the severe unmet medical needs that this would be rapidly reviewed on that, that this would expect in terms of the approval to move it up potentially after six months. So I think we're thinking about is that we would think that this can be a launch within the first half of 2016.

January 15, 2015 JPMorgan Healthcare Conference

142. On January 15, 2015, the Company participated in the JPMorgan Healthcare Conference, and discussed the development of Translarna. During the conference, Defendant Peltz commented on the "confirmatory trial" for Translarna, stating, "I expect [. . .] a number of important milestones [in 2015]. We have the confirmatory trial for Duchenne muscular dystrophy ongoing. That will allow us then to sell it in the United States, where we expect each trial to be completed this year and next year that we get approval in the US."

143. Defendant Peltz also commented on the importance of the Phase 2b trial results, stating, "And a confirmatory trial to be able to get it in the US is well underway, and it's already fully enrolled. What we did is we used the learnings from the previous study to really wring out the risk in the current study."

March 2, 2015 Form 10-K

144. On March 2, 2015, the Company filed a Form 10-K with the SEC for the fiscal quarter and year ended December 31, 2014 (the "2014 10-K"), signed by Defendants Peltz,

Kovacs, Schmertzler, Aldrich, Jacobson, Koppel, Kranda, McDonough, Renaud, Southwell, and Zeldis.

145. In the 2014 10-K, the Company touted its learnings from its previously completed trials of Translarna and how such learnings improved the Company's trial designs for ACT DMD, stating, in relevant part:

> Based on its understood mechanism of action, Translarna may have benefit in the treatment of patients with any genetic disorder that arises as a result of a nonsense mutation. The marketing authorization granted by the EC, described above, was primarily based upon the safety and efficacy results of our prior Phase 2b clinical trial of Translarna for the treatment of nmDMD. We believe that by incorporating our learnings from our completed trials in Translarna, including natural history data and our analysis, we have been able to enhance our trial designs for ACT DMD and ACT CF. We completed our Phase 2b clinical trial of Translarna for the treatment of nmDMD in 2009 and we completed a Phase 3 clinical trial of Translarna for the treatment of nmCF in 2011. While we did not achieve the primary efficacy endpoint in either trial with the pre-specified level of statistical significance, we believe that the collective data from these trials, including retrospective and subgroup analyses that we have performed, provide strong support for concluding that Translarna was active and showed clinically meaningful improvements over placebo in these trials.

146. Attached to the 2014 10-K were SOX Certifications signed by Defendants Peltz and Kovacs, attesting to the accuracy of the 2014 10-K

March 9, 2015 ROTH Capital Annual Conference

147. On March 9, 2015, the Company attended the ROTH Capital Annual Conference, discussed the positive results of the Company's Phase 2b trial and the differences of the "confirmatory" ACT DMD trial and that the latter would lead to FDA review and approval. The Company's Senior Vice President, Head of Clinical Development and Translational Research, Tuyen Ong, stated, in relevant part:

> I think we talked about the confirmatory studies really being enriched and sort of enhanced and somewhat we've wrung out the risk of the confirmatory study based on the learnings of the second study. I think ultimately it's really dependent on the data; is there a clinical benefit, is it statistically significant. So, a lot of moving factors around this. But I think based upon what we've done to address some of the

> sort of risk that we talked about, we feel pretty confident that we've addressed all the sort of items that we can take care of at this stage.

April 28, 2015 Proxy Statement

148. On April 28, 2015, the Company filed the 2015 Proxy Statement. The 2015 Proxy Statement failed to disclose the material adverse facts as discussed herein, and such misrepresentation and omissions were material to Plaintiff in voting on the matters set forth for shareholder determination in the 2015 Proxy Statement, including election of directors and appointment of an independent auditor.

149. Moreover, the 2015 Proxy Statement was false and misleading when it discussed the Company's adherence to specific governance policies and procedures, including the Code of Conduct, due to the Individual Defendants' failures to abide by them and their engagement in the scheme to issue false and misleading statements and/or omissions of material fact. The 2015 Proxy Statement stated, in relevant part:

> We have adopted a written Code of Business Conduct and Ethics, which is a code of ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. We have posted a current copy of the Code of Business Conduct and Ethics on the Corporate Governance page of the Investors section of our website, *www.ptcbio.com*. In addition, we intend to post on our website all disclosures that are required by law or NASDAQ's listing standards concerning any amendments to, or waivers from, any provision of the Code of Business Conduct and Ethics.

May 6, 2015 Deutsche Bank Health Care Conference

150. On May 6, 2015, the Company attended the Deutsche Bank Health Care Conference, where Defendant Kovacs discussed PTC's monitoring of the Phase 3 trial and the likelihood that the FDA would approve Translarna if the outcome of the ACT DMD mirrored the Phase 2b Trial. Defendant Kovacs stated, in relevant part:

> The data would actually [] have to be substantially worse than the last Phase II study to miss the 0.05 significance, if that makes any sense. . . . [O]bviously we've tried to mitigate as much risk as possible in this study by a lot of the care around reducing the enrollment criteria and try to control for the patients that were at least enrolled in the study, but in addition to that, we're trying to think about the statistical analysis plan that gets submitted in terms of what are pre-defined subgroups that we can define. In case the overall population has a near miss, can we hit it in a predefined subgroup? So we're doing that (inaudible) de-risk the outcome.

May 12, 2015 Bank of America Merrill Lynch Health Care Conference

151. On May 12, 2015, the Company attended the Bank of America Merrill Lynch Health Care Conference, where Defendant Kovacs continued to express confidence in the ACT DMD trial, stating in relevant part:

> [I]f you think about what have we done and refined for this study versus the prior Phase 2 study that gives us such a high degree of confidence in the likelihood of a positive outcome in this study later this year, one, we really refine the enrollment criteria in this study versus the last study. We take boys only seven years up to 16, whereas before, we took five and six year old boys.

October 15, 2015 Conference Call

152. On October 15, 2015, the Company hosted a conference call discussing the results of the ACT DMD Phase 3 trial. During the conference call, Defendant Peltz commented on the success of the "two large placebo-controlled trials and their demonstrated efficacy," stating, in relevant part:

> Turning to the results of ACT DMD trial on slide 5, we are very pleased that the totality of the Translarna results demonstrate clinical benefit for DMD. These include ITT results, the prespecified subgroup results, and pre-specified meta-analysis, as well as secondary timed-function tests and the North Star Assessment.

153. During the conference call, Defendant Peltz expanded on his comments of the success of the Phase 3 trial, attributing it to the "meta-analysis" that purportedly "demonstrates a clinically relevant benefit in preserving muscle function, and in changing the course of disease for

Translarna treated DMD patients across all primary and secondary endpoints over a 48-week period."

154. In further touting the results of the ACT DMD trial, Defendant Peltz stated:

> The totality of the data for Translarna demonstrates clinical benefit across primary and secondary endpoints. We have prespecified the key subgroup for analysis and the meta-analysis, both of which show Translarna had a clinically meaningful benefit for DMD patients. The results from ACT DMD trial showed consistent evidence of the clinical benefit of Translarna for individuals with nonsense mutation Duchenne muscular dystrophy, and it's impact on the course of the disorder, and the quality of life for those boys and young men.

155. During the conference call, Defendant Peltz fielded questions from numerous analysts, and emphasized that the Company had discussed with the FDA the Company's use of subgroups and suggested that the Company's statistical analyses to be submitted with its 2016 NDA had been previously approved by the FDA, stating, in relevant part:

> [Joel Beatty, Citigroup]
>
> Hi, thanks for taking the question. So have you had discussions with the FDA on the degree of consideration they might give to pre-specified meta-analysis? And if so, can you provide any more information on that?
>
> [Defendant Peltz]
>
> Sure, yes. Thanks for that question. The pre-specified metaanalysis was in our statistical analysis plan, which we had discussions with with[sic] the FDA. This was in part, part of the pre-specified plan. So they are well aware that this was agreed upon, or what was in our plan. So, yes, that's in a sense, standard procedure. I should make one other point, is that we have had conversations with the FDA. One thing they even said we could say publicly, this is very important for them, and that they are trying to rapidly, giving this a high priority, give the highest priority in order to try and get drugs to these patients.

156. Also during the conference call, Defendant Peltz noted once again that the results of the ACT DMD trial "confirmed" that Translarna purportedly treated nmDMD effectively, stating, in relevant part:

> Well, thanks all for the questions, and thanks for joining us today. We are proud to have confirmed the benefit of Translarna for the DMD patients. After over 17 years of effort, this is a rewarding moment for everyone. Again, we are very proud, and we wish you all to have a good evening. Thanks much.

November 9, 2015 Press Release, Form 10-Q & Conference Call

157. On November 9, 2015, the Company issued a press release announcing the Company's financial results for the fiscal quarter ended September 30, 2015 and providing updates on ACT DMD. In the press release, the Company expressed confidence regarding the FDA review and potential approval of Translarna for nmDMD, stating, in relevant part:

> ACT DMD results confirm clinical benefit of Translarna in nonsense mutation Duchenne muscular dystrophy. On October 15th, PTC announced results from the Phase 3 ACT DMD clinical trial of Translarna in patients with nmDMD. The totality of the clinical data from two large, placebo-controlled clinical trials across over 400 patients demonstrates Translarna's ability to slow disease progression. Today, on PTC's quarterly investor call the Company will review key findings from the ACT DMD clinical trial.

158. Also on November 9, 2015 the Company filed a Form 10-Q with the SEC for the fiscal quarter ended September 30, 2015 (the "3Q 2015 10-Q"), signed by Defendant Kovacs. The 3Q 2015 10-Q commented on the purported "substantial evidence of the effectiveness of Translarna," stating, in relevant part:

> [W]e believe that the results of ACT DMD and the totality of clinical data across our two large, randomized, placebo-controlled trials (ACT DMD and our prior Phase 2b study, Study 007), provide substantial evidence of the effectiveness of Translarna and demonstrate a clinically meaningful benefit of Translarna for the treatment of nmDMD.

159. Attached to the 3Q 2015 10-Q were SOX Certifications signed by Defendants Peltz and Kovacs, attesting to the accuracy of the 3Q 2015 10-Q.

160. The Company also held an earnings call on November 9, 2015 to discuss its financial results for the third quarter 2015. During the call, Defendant Peltz suggested that Translarna had demonstrated efficacy that would be sufficient for approval by the FDA, stating,

in relevant part, "The goal is to show efficacy with given endpoints in the limited window of a 48-week clinical study. We see this in ACT DMD. . . . . the totality of clinical data confirmed Translarna's ability to slow disease progression for patients with DMD."

November 18, 2015 Stifel Healthcare Conference

161. On November 18, 2015, the Company attended the Stifel Healthcare Conference, where Defendant Kovacs expressed confidence that Translarna would be reviewed quickly and subsequently approved by the FDA, stating, in relevant part:

> And the big picture about our data is and what will be part of our argument to both the regulatory authorities in the US and Europe is that the consistency of the results now seen across two of the largest placebo-controlled Phase 3 studies ever done in the disease, the totality of the data support the clinical benefit and certainly the risk-benefit profile of the drug in favor of an approval and getting something to these kids.

December 9, 2015 Oppenheimer Healthcare Conference

162. On December 9, 2015, the Company attended the Oppenheimer Healthcare Conference, where Defendant Kovacs discussed the Company's intention to file for full approval, stating, in relevant part, "Our intention today is for filing for full approval on the basis of two large well-controlled studies that all point to safety and efficacy for a risk-benefit profile in favor of the drug."

January 13, 2016 JPMorgan Healthcare Conference

163. On January 13, 2016, the Company attended the JPMorgan Healthcare Conference, where Defendant Peltz, based on the results of the ACT DMD, expressed continued confidence in the likelihood of FDA approval of Translarna for nmDMD, stating, in relevant part:

> So you can see in two large studies where we used the six-minute walk test as the primary endpoint, we saw a benefit both in the primary endpoint as well as secondary endpoints. And in prespecified subgroups, we saw more robust effects being observed, both the primary and secondary endpoints. So consistent data in two independent studies.

> One of the things we've noticed they asked for was a sensitivity analysis, and that while you have prespecified subgroups, if you go beyond those, does the data still show clinically meaningful differences? And it does both in the primary and secondary endpoints.

164. Also during the conference, Defendant Peltz commented on the Company's meta-analysis and the consistency of both the Phase 2b and Phase 3 trials in demonstrating the efficacy of Translarna, stating, in relevant part:

> In the meta-analysis, where you combine the results, you see both in the six-minute walk distance as well as the time function tests, you see clinically meaningful and statistically significant improvements with Translarna over placebo. So, when you look at -- and I think here's a nice example, I'm looking at the forest plot, looking at the totality of the results, you see quite clearly that treating patients with Translarna always was better – always showed efficacy versus placebo, no matter whether you look at the six-minute walk test or the time function test.
>
> And really it's consistent with the totality of the data, demonstrating that this drug was efficacious. So, I think we've checked that box.

The Truth Emerges

February 23, 2016 Press Release

165. On February 23, 2016, the Company issued a press release announcing that the FDA had issued an RTF to the Company for the 2016 NDA, meaning that the FDA would not be reviewing any data in the 2016 NDA and was rejecting it outright for being inadequate on its face.

166. On this news, the price per share of PTC stock fell $17.42 per share, or approximately 61.6%, from the previous day's closing price to close at $10.84 on February 23, 2016.

February 29, 2016 Press Release

167. On February 29, 2016, the Company issued a press release revealing additional details regarding the RTF letter for Translarna for the treatment of nmDMD, including the lack of "substantial evidence of effectiveness" of both the Phase 2b and Phase 3 trials and that "certain of

the company's adjustments to the ACT DMD study [were] post hoc and therefore not supportive of effectiveness." The press release stated, in relevant part:

> **Refuse to File letter received from the FDA regarding Translarna for nonsense mutation Duchenne muscular dystrophy (nmDMD).** The letter from the U.S. Food and Drug Administration (FDA) received on February 22, 2016 stated that the new drug application (NDA) for Translarna was not sufficiently complete to permit a substantive review. Specifically, PTC was notified in the letter that, in the view of the FDA, both the Phase 2b and ACT DMD trials were negative and do not provide substantial evidence of effectiveness. The FDA also characterized certain of the company's adjustments to the ACT DMD study as post hoc and therefore not supportive of effectiveness. In addition, the FDA noted that the NDA did not contain adequate information regarding the abuse potential of Translarna, a requirement for new molecules that cross the blood-brain barrier. PTC is engaging in dialogue with the FDA to discuss and clarify the matters set forth in the letter and to determine the best path forward.

February 29, 2016 Earnings Call

168. On February 29, 2016, the Company held an earnings call discussing its financial results for the fourth quarter and fiscal year ended December 31, 2015 and also the RTF letter. During the earnings call, Defendant Peltz noted that the FDA believed the 2016 NDA was insufficient because "relying on the [300-400 meter] subgroup as the main analysis is considered as a post hoc adjustment," and that the submission by PTC therefore "eliminates data from a majority of enrolled patients." Peltz also noted, with regard to the FDA's view, "in the view of the FDA, both the Phase IIB and Phase III ACT DMD trials were negative and did not provide substantial evidence of effectiveness. The FDA also characterized certain of our adjustments to the ACT DMD study as post-hoc and therefore not supportive of effectiveness."

169. During the earnings call, Defendant Peltz was asked about the FDA's characterization of certain analyses as post hoc, with an analyst noting that PTC's past comments indicated "that the stat plan was submitted to the FDA earlier in 2015," to which Defendant Peltz

responded by noting that the FDA never commented on subgroups although the Company had submitted a statistical analysis plan which included subgroups. Peltz stated, in relevant part:

> On the -- overall on the subgroup analysis, the FDA characterized -- first of all the FDA characterized our NDA as not supportive of effectiveness. This would include the subgroup analysis.
>
> So I think in terms of the history it's important to remember that we included the subgroup in our statistical analysis plan which we submitted in the spring of 2015. At that time the FDA commented on our statistical analysis plan but had no comments on our subgroups.
>
> We submitted the final statistical analysis plan to the FDA before unblinding the ACT DMD study. However in the RTF letter the FDA characterized that PTC proposed a post hoc adjustment of ACT DMD that eliminates data from a majority of enrolled patients. So we need further discussion with the FDA to understand their current perspective on our subgroup analysis.
>
> We believe the FDA's perspective in the RTF letter may be that although we've pre-specified the subgroup, relying on the subgroup as the main analysis is considered as a post hoc adjustment and we'll be talking to them further on this point.

170. Several analysts responded to the news by downgrading PTC's stock and citing the Company's eroded credibility and lack of transparency. An analyst report titled "Downgrading As Translarna Becomes a Show-Me Story," in particular, highlighted the level of miscommunication between the Company and the FDA and the fact that the 2016 NDA was rejected despite the substantial leeway given to orphan-drug developers by the FDA, stating, in relevant part:

> [T]he issue was the fact that FDA deemed the baseline 6MWT subpopulation analysis as "post-hoc" or otherwise deficient in statistical-meaning. PTCT does assert that it submitted the subpopulation analysis in its statistical analysis plan, which FDA did not comment on. We note, again, that PTCT did not meet with FDA after ACT-DMD results were released and before completion of the rolling NDA. The level of miscommunication (or lack of communication) with the agency suggests a major breakdown of the regulatory communication process. It is also at odds with FDA's very public message to orphan drug developers to talk to them "early and often."

> Our concern around the lack of quality communication is only deepened by the second point of the RTF: the inadequacy of the abuse potential. We believe this an issue addressed by a large proportion of sponsors, and is therefore a relatively straightforward, almost mundane, regulatory requirement that somehow slipped through the cracks of the Translarna filing package.

Appeal of the RTF Letter

171. On July 25, 2016, the Company issued a press release stating that it had "recently submitted an appeal to escalate continuing discussions about the RTF decision to the next level of FDA management via the formal dispute resolution process." On October 17, 2016, the Company announced via press release that the Office of Drug Evaluation I of the FDA "denied the company's first appeal of the refuse to file letter" for the NDA for Translarna, and that PTC "intends to escalate its appeal to the next supervisory level of the FDA."

March 6, 2017 Press Release

172. On March 6, 2017, the Company issued a press release announcing that the FDA had acknowledged the filing-over-protest of the NDA for Translarna. The press release noted that "PTC used the FDA's file over protest regulations to file the NDA," which "allow a company to have its NDA filed and reviewed following receipt of a refuse to file determination."

June 6, 2017 Press Release

173. On June 6, 2017, the Company issued a press release announcing that the FDA had notified the Company of the Advisory Committee Meeting with the PCNSDAC, scheduled for September 28, 2017, to review the filed-over-protest NDA for Translarna. The press release also noted that the FDA had set a goal date of October 24, 2017 for completion of its review of the NDA for Translarna.

Advisory Committee Meeting Briefing Materials

174. On September 26, 2017, the PCNSDAC published briefing materials in advance of the Advisory Committee Meeting, which reaffirmed the failures of the NDA for Translarna, however in much greater detail and to further extent. The briefing materials meticulously analyzed the data provided in the NDA for Translarna, citing issues with not only the data itself, but also the presentation of it. The briefing materials ultimately noted that, "Overall, the data intended by the applicant to establish the effectiveness of ataluren for the treatment of nmDMD are not persuasive."

175. The briefing materials stated, in relevant part:

> The application contains a large number of exploratory analyses that lack interpretability and are often entirely based on unblinded data. The presentation of the data in the application is often unclear as to which analyses were used by the applicant. Ultimately, no positive results from any prospectively planned analyses that are persuasive have been provided with this application. In the one instance where an exploratory analysis (the unblinded post hoc analysis of Study 007) was prospectively tested (in Study 020), the results were clearly negative.
>
> * * *
>
> Ultimately, no positive results from any prospectively planned analyses that are persuasive have been provided with this application. The applicant has presented only the results from numerous post hoc and exploratory analyses that are intended to mitigate two negative clinical trials. In 2011, the applicant claimed that the effectiveness of ataluren had been established based on the post hoc analyses of the ADP population in Study 007. However, when this conclusion was prospectively evaluated in Study 020, the results were clearly negative. This finding directly highlights the frequently misleading nature of exploratory analyses of negative trials.

176. As noted by *STAT* in a September 26, 2017 article titled "FDA rips into PTC's Duchenne muscular dystrophy drug as advisory panel looms," "Translarna was rather robustly savaged" in the review.

177. On this news, the price per share of PTC stock fell $2.70 per share, or approximately 13.8%, from the previous day's closing price to close at $16.81 on September 26, 2017.

The Advisory Committee Meeting

178. On September 28, 2017, 10 of the 11 members of the PCNSDAC endorsed the opinion in the Advisory Committee Meeting briefing materials that data to establish effectiveness of Translarna "are not persuasive." As noted in PTC's Form 8-K filed with the SEC on September 28, 2017, 10 members of the PCNSDAC panel specifically voted that "although it is possible that [Translarna] may be effective, the data are inconclusive, and more work would be needed to establish whether [Translarna] is effective," a far cry from the likelihood of success portrayed by the Individual Defendants during the Relevant Period.

179. Although the outcome for the Translarna NDA won't be revealed until October 24, 2017, *Reuters* noted in a September 28, 2017 article that "[t]he panel's vote reduces the chance the drug will be approved soon since the FDA generally follows the advice of its advisors." Thus, despite the data supporting the NDA for Translarna being repeatedly rejected, and the already low likelihood of its approval, the Individual Defendants continue to push for approval of an NDA that going forward has an even more miniscule likelihood of approval.

180. The statements referenced above in ¶¶ 139-165 were materially false and misleading because they misrepresented and failed to disclose material adverse facts pertaining to the Company's business, operations, prospects, and legal compliance, which were known to the Individual Defendants or recklessly disregarded by them. Specifically, the Individual Defendants willfully or recklessly made and/or caused the Company to make false and/or misleading statements and/or omissions of material fact that failed to disclose:

(1) The substantial risk that the NDA submission for Translarna would be rejected by the FDA as facially insufficient and that the ACT DMD trial would not meet its primary clinical endpoints, thus resulting in the FDA refusing to file the 2016 NDA for

substantive review and only ultimately reviewing it because it was filed-over-protest by the Company;

(2) PTC would be required by the FDA to demonstrate the efficacy of Translarna more sufficiently than PTC had done in the Phase 2b trials, and the ACT DMD trial's portrayal as "confirmatory" was materially misleading;

(3) The substantial likelihood that the 2016 NDA would not be reviewed at all by the FDA if the results of the ACT DMD trial did not "confirm" Translarna's efficacy as demonstrated purportedly by the Phase 2b trial's results;

(4) There was just as much of a risk of failure in the design of the ACT DMD trial as was present in the Phase 2b trial, any risks of negative outcomes were not lessened by the design of the ACT DMD, and there was no basis to believe that the outcomes of the ACT DMD trial would positively demonstrate efficacy as determined by the trial's primary clinical endpoints;

(5) The Phase 3 trial results were less supportive of a finding of efficacy than the results drawn from the Phase 2b trial—and thus did not "confirm" the benefit for DMD patients in using Translarna—and this was facially insufficient to support substantive review by the FDA;

(6) The claimed efficacy of Translarna was supported by meta-analyses results that were only applicable to a small subgroup of nmDMD patients and not pre-specified in PTC's statistical analysis plan, meaning that the meta-analyses would be facially insufficient to form a complete application that the FDA would file;

(7) The FDA would consider the Company's reliance on the 300-400 meter subgroup as the main analysis to be a post-hoc adjustment, and that the Company's ultimate submission

discarded data from a majority of the Phase 3 trial's patients, thus rendering inadequate on its face the 2016 NDA submission;

(8) The risk of the 2016 NDA being facially insufficient to support an approval for Translarna's broad label use for all nmDMD patients was underrepresented, and that the Individual Defendants in fact knew that only showing statistical significance for a small subgroup and in meta-analyses would be facially insufficient to support a complete application that the FDA would review;

(9) The statements made by the Defendants suggesting that the Company's gathered data was sufficient to support substantive review or FDA approval were contradicted by additional efficacy requirements relayed to PTC in discussions with the FDA after PTC received the RTF letter for its 2011 NDA and in guidance provided to the Company from the FDA regarding the development of drugs for the treatment of DMD;

(10) The primary clinical endpoints and other ITT analyses of the ACT DMD trial were not met, and thus the trial did not demonstrate to a statistically significant level a clinically meaningful benefit of treatment;

(11) Ultimately, the ACT DMD trial's data would be facially insufficient to form a complete application that would be reviewed by the FDA without the Company filing it over protest; and

(12) The Company failed to maintain internal controls.

Summary of Individual Defendants' Misconduct

181. In breach of their fiduciary duties, the Individual Defendants willfully or recklessly caused or permitted the Company to make the false and misleading statements and omissions of material fact to the investing public as set forth above.

182. Moreover, the Individual Defendants failed to correct and/or caused the Company to fail to correct the false and/or misleading statements and/or omissions of material fact referenced herein, rendering them personally liable to the Company for breaching their fiduciary duties.

183. Additionally, while the Individual Defendants caused the Company's stock to be artificially inflated, six of the Individual Defendants benefitted themselves by engaging in insider sales.

184. In further breach of their fiduciary duties, the Individual Defendants failed to maintain adequate internal controls.

DAMAGES TO PTC

185. As a direct and proximate result of the Individual Defendants' conduct, PTC will lose and expend many millions of dollars.

186. Such expenditures include, but are not limited to, legal fees associated with the Securities Class Action filed against the Company, its CEO, and its CFO, and any internal investigations, and amounts paid to outside lawyers, accountants, and investigators in connection thereto.

187. Additionally, these expenditures include, but are not limited to, lavish compensation and benefits paid to the Individual Defendants who breached their fiduciary duties to the Company.

188. As a direct and proximate result of the Individual Defendants' conduct, PTC has also suffered and will continue to suffer a loss of reputation and goodwill, and a "liar's discount" that will plague the Company's stock in the future due to the Company's and their misrepresentations and the Individual Defendants' breaches of fiduciary duties and unjust enrichment.

DERIVATIVE ALLEGATIONS

189. Plaintiff brings this action derivatively and for the benefit of PTC to redress injuries suffered, and to be suffered, as a result of the Individual Defendants' breaches of their fiduciary duties as directors and/or officers of PTC, gross mismanagement, abuse of control, waste of corporate assets, unjust enrichment, violations of Section 14(a) of the Exchange Act, as well as the aiding and abetting thereof.

190. PTC is named solely as a nominal party in this action. This is not a collusive action to confer jurisdiction on this Court that it would not otherwise have.

191. Plaintiff is, and has been at all relevant times, a shareholder of PTC. Plaintiff will adequately and fairly represent the interests of PTC in enforcing and prosecuting its rights, and, to that end, has retained competent counsel, experienced in derivative litigation, to enforce and prosecute this action.

DEMAND FUTILITY ALLEGATIONS

192. Plaintiff incorporates by reference and re-alleges each and every allegation stated above as if fully set forth herein.

193. A pre-suit demand on the Board of PTC is futile and, therefore, excused. At the time of filing of this action, the Board consists of the following seven individuals: Defendants Peltz, Schmertzler, Jacobson, Southwell, Steele, and Zeldis (collectively, the "Director-Defendants"), and non-defendant Dawn Svoronos (together with the Director-Defendants, the "Directors"). Plaintiff needs only to allege demand futility as to four of the seven directors who are on the Board at the time this action is commenced.

194. Demand is excused as to all of the Director-Defendants because each one of them faces, individually and collectively, a substantial likelihood of liability as a result of the scheme

they engaged in knowingly or recklessly to make and/or cause the Company to make false and misleading statements and omissions of material facts, while three of the Director-Defendants engaged in insider sales, which renders them unable to impartially investigate the charges and decide whether to pursue action against themselves and the other perpetrators of the scheme.

195. In complete abdication of their fiduciary duties, the Director-Defendants either knowingly or recklessly participated in making and/or causing the Company to make the materially false and misleading statements alleged herein. The fraudulent scheme was intended to make the Company appear more profitable and attractive to investors. While investors were duped into believing the fraud perpetrated by the Individual Defendants, six of the Individual Defendants collectively sold over $19.8 million worth of Company stock at artificially inflated prices based on material inside information. As a result of the foregoing, the Director-Defendants breached their fiduciary duties, face a substantial likelihood of liability, are not disinterested, and demand upon them is futile, and thus excused.

196. Additional reasons that demand on Defendant Peltz is futile follow. Defendant Peltz currently serves as the Company's CEO, and is thus, as the Company admits, a non-independent director. He received lavish compensation, including $10,518,723 in 2015. Defendant Peltz was ultimately responsible for all of the false and misleading statements and omissions that were made, including those contained in the SEC filings, press releases, and conferences calls referenced herein, the vast majority of which he personally made statements in. His large Company stock holding, worth over $26.2 million before the fraud was exposed, reveals his interest in keeping the Company's stock price as high as possible. As the Company's highest officer and as a trusted Company director at all relevant times, he conducted little, if any, oversight of the Company's engagement in the scheme to make false and misleading statements, consciously

disregarded his duties to monitor such controls over reporting and engagement in the scheme, and consciously disregarded his duties to protect corporate assets. Moreover, Defendant Peltz is a defendant in the Securities Class Action. Defendant Peltz's insider sales before the fraud was exposed, which yielded over $12.1 in proceeds, demonstrates his motive in facilitating and participating in the fraud. Furthermore, Defendant Peltz's domestic partner is employed by the Company and his brother is a principal for a company that provides IT, tax, and audit services to PTC, and Peltz may fear retaliation against them, in addition to himself, if he accepts Plaintiff's demand. For these reasons, too, Defendant Peltz breached his fiduciary duties, faces a substantial likelihood of liability, is not independent or disinterested, and thus demand upon him is futile and, therefore, excused.

197. Additional reasons that demand on Defendant Schmertzler is futile follow. Defendant Schmertzler has served as a Company director since 2001 and as Chair of the Board since 2004. He is also Chair of the Nominating and Corporate Governance Committee and a member of the Compensation Committee. He received lavish compensation, including $770,602 in 2015. His large Company stock holding, worth over $176.3 million before the fraud was exposed, reveals his interest in keeping the Company's stock price as high as possible. As a long-time director, he conducted little, if any, oversight of the Company's engagement in the scheme to make false and misleading statements, consciously disregarded his duties to monitor such controls over reporting and engagement in the scheme, and consciously disregarded his duties to protect corporate assets. Moreover, he made false and misleading statements himself, as he signed the 2014 10-K. Thus, for these reasons, too, Defendant Schmertzler breached his fiduciary duties, faces a substantial likelihood of liability, is not independent or disinterested, and thus demand upon him is futile and, therefore, excused.

198. Additional reasons that demand on Defendant Jacobson is futile follow. Defendant Jacobson has served as a Company director since 1998 and is a co-founder of the Company. He received lavish compensation, including $487,801 in 2015. His large Company stock holding, worth over $4.4 million before the fraud was exposed, reveals his interest in keeping the Company's stock price as high as possible. As a long-time director, he conducted little, if any, oversight of the Company's engagement in the scheme to make false and misleading statements, consciously disregarded his duties to monitor such controls over reporting and engagement in the scheme, and consciously disregarded his duties to protect corporate assets. Moreover, he made false and misleading statements himself, as he signed the 2014 10-K. Defendant Jacobson's insider sales before the fraud was exposed, which yielded over $1.5 million in proceeds, demonstrates his motive in facilitating and participating in the fraud. Thus, for these reasons, too, Defendant Jacobson breached his fiduciary duties, faces a substantial likelihood of liability, is not independent or disinterested, and thus demand upon him is futile and, therefore, excused.

199. Additional reasons that demand on Defendant Southwell is futile follow. Defendant Southwell has served as a Company director since 2005. He is also Chair of the Compensation Committee and a member of the Audit Committee. He received lavish compensation, including $417,801 in 2015. His large Company stock holding, worth over $2.7 million before the fraud was exposed, reveals his interest in keeping the Company's stock price as high as possible. As a long-time director and member of the Audit Comittee, he conducted little, if any, oversight of the Company's engagement in the scheme to make false and misleading statements, consciously disregarded his duties to monitor such controls over reporting and engagement in the scheme, and consciously disregarded his duties to protect corporate assets. Moreover, he made false and misleading statements himself, as he signed the 2014 10-K.

Defendant Southwell's insider sales before the fraud was exposed, which yielded over $1.2 million in proceeds, demonstrates his motive in facilitating and participating in the fraud. Thus, for these reasons, too, Defendant Southwell breached his fiduciary duties, faces a substantial likelihood of liability, is not independent or disinterested, and thus demand upon him is futile and, therefore, excused.

200. Additional reasons that demand on Defendant Zeldis is futile follow. Defendant Zeldis has served as a Company director since September 2012. He is also member of the Nominating and Corporate Governance Committee. He received lavish compensation, including $394,801 in 2015. As a long-time director, he conducted little, if any, oversight of the Company's engagement in the scheme to make false and misleading statements, consciously disregarded his duties to monitor such controls over reporting and engagement in the scheme, and consciously disregarded his duties to protect corporate assets. Moreover, he made false and misleading statements himself, as he signed the 2014 10-K. Thus, for these reasons, too, Defendant Zeldis breached his fiduciary duties, faces a substantial likelihood of liability, is not independent or disinterested, and thus demand upon him is futile and, therefore, excused.

201. Additional reasons that demand on Defendant Steele is futile follow. Defendant Steele has served as a Company director since June 2015. He is also a member of the Compensation Committee. He received lavish compensation, including $680,632 in 2015. As a long-time director, he conducted little, if any, oversight of the Company's engagement in the scheme to make false and misleading statements, consciously disregarded his duties to monitor such controls over reporting and engagement in the scheme, and consciously disregarded his duties to protect corporate assets. Thus, for these reasons, too, Defendant Steele breached his fiduciary

duties, faces a substantial likelihood of liability, is not independent or disinterested, and thus demand upon him is futile and, therefore, excused.

202. Additional reasons that demand on the Board is futile follow.

203. The Directors have longstanding business and personal relationships with each other and the Individual Defendants that preclude them from acting independently and in the best interests of the Company and the shareholders. In fact, three of the Directors have served on the Board for over a decade. These conflicts of interest precluded the Directors from adequately monitoring the Company's operations and internal controls and calling into question the Individual Defendants' conduct. Thus, demand upon the Directors would be futile.

204. In violation of the Code of Conduct, the Director-Defendants conducted little, if any, oversight of the Company's engagement in the Individual Defendants' scheme to issue materially false and misleading statements to the public and to facilitate and disguise the Individual Defendants' violations of law, including breaches of fiduciary duty, gross mismanagement, abuse of control, waste of corporate assets, unjust enrichment, and violations of Section 14(a) of the Exchange Act. In violation of the Code of Conduct, the Director-Defendants failed to comply with laws and regulations, maintain the accuracy of Company records and reports, and uphold the employee and director responsibilities related thereto. Thus, the Director-Defendants face a substantial likelihood of liability and demand is futile as to them.

205. PTC has been and will continue to be exposed to significant losses due to the wrongdoing complained of herein, yet the Directors have not filed any lawsuits against themselves or others who were responsible for that wrongful conduct to attempt to recover for PTC any part of the damages PTC suffered and will continue to suffer thereby. Thus, any demand upon the Directors would be futile.

206. The Individual Defendants' conduct described herein and summarized above could not have been the product of legitimate business judgment as it was based on bad faith and intentional, reckless, or disloyal misconduct. Thus, none of the Director-Defendants can claim exculpation from their violations of duty pursuant to the Company's charter (to the extent such a provision exists). As a majority of the Director-Defendants face a substantial likelihood of liability, they are self-interested in the transactions challenged herein and cannot be presumed to be capable of exercising independent and disinterested judgment about whether to pursue this action on behalf of the shareholders of the Company. Accordingly, demand is excused as being futile.

207. The acts complained of herein constitute violations of fiduciary duties owed by PTC's officers and directors, and these acts are incapable of ratification.

208. The Directors may also be protected against personal liability for their acts of mismanagement and breaches of fiduciary duty alleged herein by directors' and officers' liability insurance if they caused the Company to purchase it for their protection with corporate funds, i.e., monies belonging to the stockholders of PTC. If there is a directors' and officers' liability insurance policy covering the Directors, it may contain provisions that eliminate coverage for any action brought directly by the Company against the Directors, known as, *inter alia*, the "insured-versus-insured exclusion." As a result, if the Directors were to sue themselves or certain of the officers of PTC, there would be no directors' and officers' insurance protection. Accordingly, the Directors cannot be expected to bring such a suit. On the other hand, if the suit is brought derivatively, as this action is brought, such insurance coverage, if such an insurance policy exists, will provide a basis for the Company to effectuate a recovery. Thus, demand on the Directors is futile and, therefore, excused.

209. If there is no directors' and officers' liability insurance, then the Directors will not cause PTC to sue the Individual Defendants named herein, since, if they did, they would face a large uninsured individual liability. Accordingly, demand is futile in that event, as well.

210. Thus, for all of the reasons set forth above, all of the Directors, and, if not all of them, certainly at least four of the Directors, cannot consider a demand with disinterestedness and independence. Consequently, a demand upon the Board is excused as futile.

FIRST CLAIM

Against Individual Defendants for Violations of Section 14(a) of the Securities Exchange Act of 1934

211. Plaintiff incorporates by reference and re-alleges each and every allegation set forth above, as though fully set forth herein.

212. Section 14(a) of the Exchange Act, 15 U.S.C. § 78n(a)(1), provides that "[i]t shall be unlawful for any person, by use of the mails or by any means or instrumentality of interstate commerce or of any facility of a national securities exchange or otherwise, in contravention of such rules and regulations as the [SEC] may prescribe as necessary or appropriate in the public interest or for the protection of investors, to solicit or to permit the use of his name to solicit any proxy or consent or authorization in respect of any security (other than an exempted security) registered pursuant to section 12 of this title [15 U.S.C. § 78l]."

213. Rule 14a-9, promulgated pursuant to § 14(a) of the Exchange Act, provides that no proxy statement shall contain "any statement which, at the time and in the light of the circumstances under which it is made, is false or misleading with respect to any material fact, or which omits to state any material fact necessary in order to make the statements therein not false or misleading." 17 C.F.R. § 240.14a-9.

214. Under the direction and watch of the Directors, the 2015 Proxy Statement failed to disclose:

(1) The substantial risk that the NDA submission for Translarna would be rejected by the FDA as facially insufficient and that the ACT DMD trial would not meet its primary clinical endpoints, thus resulting in the FDA refusing to file the 2016 NDA for substantive review and only ultimately reviewing it because it was filed-over-protest by the Company;

(2) PTC would be required by the FDA to demonstrate the efficacy of Translarna more sufficiently than PTC had done in the Phase 2b trials, and the ACT DMD trial's portrayal as "confirmatory" was materially misleading;

(3) The substantial likelihood that the 2016 NDA would not be reviewed at all by the FDA if the results of the ACT DMD trial did not "confirm" Translarna's efficacy as demonstrated purportedly by the Phase 2b trial's results;

(4) There was just as much of a risk of failure in the design of the ACT DMD trial as was present in the Phase 2b trial, any risks of negative outcomes were not lessened by the design of the ACT DMD, and there was no basis to believe that the outcomes of the ACT DMD trial would positively demonstrate efficacy as determined by the trial's primary clinical endpoints;

(5) The Phase 3 trial results were less supportive of a finding of efficacy than the results drawn from the Phase 2b trial—and thus did not "confirm" the benefit for DMD patients in using Translarna—and this was facially insufficient to support substantive review by the FDA;

(6) The claimed efficacy of Translarna was supported by meta-analyses results that were only applicable to a small subgroup of nmDMD patients and not pre-specified in PTC's statistical analysis plan, meaning that the meta-analyses would be facially insufficient to form a complete application that the FDA would file;

(7) The FDA would consider the Company's reliance on the 300-400 meter subgroup as the main analysis to be a post-hoc adjustment, and that the Company's ultimate submission discarded data from a majority of the Phase 3 trial's patients, thus rendering inadequate on its face the 2016 NDA submission;

(8) The risk of the 2016 NDA being facially insufficient to support an approval for Translarna's broad label use for all nmDMD patients was underrepresented, and that the Individual Defendants in fact knew that only showing statistical significance for a small subgroup and in meta-analyses would be facially insufficient to support a complete application that the FDA would review;

(9) The statements made by the Defendants suggesting that the Company's gathered data was sufficient to support substantive review or FDA approval were contradicted by additional efficacy requirements relayed to PTC in discussions with the FDA after PTC received the RTF letter for its 2011 NDA and in guidance provided to the Company from the FDA regarding the development of drugs for the treatment of DMD;

(10) The primary clinical endpoints and other ITT analyses of the ACT DMD trial were not met, and thus the trial did not demonstrate to a statistically significant level a clinically meaningful benefit of treatment;

(11) Ultimately, the ACT DMD trial's data would be facially insufficient to form a complete application that would be reviewed by the FDA without the Company filing it over protest; and

(12) The Company failed to maintain internal controls.

215. Moreover, the 2015 Proxy Statement was false and misleading when it discussed the Company's adherence to specific governance policies and procedures, including the Code of Conduct, due to the Individual Defendants' failures to abide by them and their engagement in the scheme to issue false and misleading statements and/or omissions of material fact.

216. In the exercise of reasonable care, the Individual Defendants should have known that by misrepresenting or failing to disclose the foregoing material facts, the statements contained in the 2015 Proxy Statement were materially false and misleading. The misrepresentations and omissions were material to Plaintiff in voting on the matters set forth for shareholder determination in the 2015 Proxy Statement, including election of directors and appointment of an independent auditor.

217. The Company was damaged as a result of the Individual Defendants' material misrepresentations and omissions in the 2015 Proxy Statement.

218. Plaintiff on behalf of PTC has no adequate remedy at law.

SECOND CLAIM

Against the Individual Defendants for Breach of Fiduciary Duties

219. Plaintiff incorporates by reference and re-alleges each and every allegation set forth above, as though fully set forth herein.

220. Each Individual Defendant owed to the Company the duty to exercise candor, good faith, and loyalty in the management and administration of PTC's business and affairs.

221. Each of the Individual Defendants violated and breached his or her fiduciary duties of candor, good faith, loyalty, reasonable inquiry, oversight, and supervision.

222. The Individual Defendants' conduct set forth herein was due to their intentional or reckless breach of the fiduciary duties they owed to the Company, as alleged herein. The Individual Defendants intentionally or recklessly breached or disregarded their fiduciary duties to protect the rights and interests of PTC.

223. In breach of their fiduciary duties owed to PTC, the Individual Defendants willfully or recklessly made and/or caused the Company to make false and/or misleading statements and/or omissions of material fact that failed to disclose:

(1) The substantial risk that the NDA submission for Translarna would be rejected by the FDA as facially insufficient and that the ACT DMD trial would not meet its primary clinical endpoints, thus resulting in the FDA refusing to file the 2016 NDA for substantive review and only ultimately reviewing it because it was filed-over-protest by the Company;

(2) PTC would be required by the FDA to demonstrate the efficacy of Translarna more sufficiently than PTC had done in the Phase 2b trials, and the ACT DMD trial's portrayal as "confirmatory" was materially misleading;

(3) The substantial likelihood that the 2016 NDA would not be reviewed at all by the FDA if the results of the ACT DMD trial did not "confirm" Translarna's efficacy as demonstrated purportedly by the Phase 2b trial's results;

(4) There was just as much of a risk of failure in the design of the ACT DMD trial as was present in the Phase 2b trial, any risks of negative outcomes were not lessened by the design of the ACT DMD, and there was no basis to believe that the outcomes of the ACT

DMD trial would positively demonstrate efficacy as determined by the trial's primary clinical endpoints;

(5) The Phase 3 trial results were less supportive of a finding of efficacy than the results drawn from the Phase 2b trial—and thus did not "confirm" the benefit for DMD patients in using Translarna—and this was facially insufficient to support substantive review by the FDA;

(6) The claimed efficacy of Translarna was supported by meta-analyses results that were only applicable to a small subgroup of nmDMD patients and not pre-specified in PTC's statistical analysis plan, meaning that the meta-analyses would be facially insufficient to form a complete application that the FDA would file;

(7) The FDA would consider the Company's reliance on the 300-400 meter subgroup as the main analysis to be a post-hoc adjustment, and that the Company's ultimate submission discarded data from a majority of the Phase 3 trial's patients, thus rendering inadequate on its face the 2016 NDA submission;

(8) The risk of the 2016 NDA being facially insufficient to support an approval for Translarna's broad label use for all nmDMD patients was underrepresented, and that the Individual Defendants in fact knew that only showing statistical significance for a small subgroup and in meta-analyses would be facially insufficient to support a complete application that the FDA would review;

(9) The statements made by the Defendants suggesting that the Company's gathered data was sufficient to support substantive review or FDA approval were contradicted by additional efficacy requirements relayed to PTC in discussions with the FDA after PTC

received the RTF letter for its 2011 NDA and in guidance provided to the Company from the FDA regarding the development of drugs for the treatment of DMD;

(10) The primary clinical endpoints and other ITT analyses of the ACT DMD trial were not met, and thus the trial did not demonstrate to a statistically significant level a clinically meaningful benefit of treatment;

(11) Ultimately, the ACT DMD trial's data would be facially insufficient to form a complete application that would be reviewed by the FDA without the Company filing it over protest; and

(12) The Company failed to maintain internal controls.

224. The Individual Defendants also failed to correct and/or caused the Company to fail to correct the false and/or misleading statements and/or omissions of material fact, rendering them personally liable to the Company for breaching their fiduciary duties.

225. Also in breach of their fiduciary duties, the Individual Defendants failed to maintain internal controls.

226. Additionally, six of the Individual Defendants engaged in lucrative insider sales while the price of the Company's common stock was artificially inflated due to the false and misleading statements of material fact referenced herein.

227. The Individual Defendants had actual or constructive knowledge that the Company issued materially false and misleading statements, and they failed to correct the Company's public statements and representations. The Individual Defendants had actual knowledge of the misrepresentations and omissions of material facts set forth herein, or acted with reckless disregard for the truth, in that they failed to ascertain and to disclose such facts, even though such facts were available to them. Such material misrepresentations and omissions were committed knowingly or

recklessly and for the purpose and effect of artificially inflating the price of PTC's securities and disguising insider sales.

228. The Individual Defendants had actual or constructive knowledge that they had caused the Company to improperly engage in the fraudulent schemes set forth herein and to fail to maintain internal controls. The Individual Defendants had actual knowledge that the Company was engaging in the fraudulent schemes set forth herein, and that internal controls were not adequately maintained, or acted with reckless disregard for the truth, in that they caused the Company to improperly engage in the fraudulent schemes and to fail to maintain adequate internal controls, even though such facts were available to them. Such improper conduct was committed knowingly or recklessly and for the purpose and effect of artificially inflating the price of PTC's securities and engaging in insider sales.

229. These actions were not a good-faith exercise of prudent business judgment to protect and promote the Company's corporate interests.

230. As a direct and proximate result of the Individual Defendants' breaches of their fiduciary obligations, PTC has sustained and continues to sustain significant damages. As a result of the misconduct alleged herein, the Individual Defendants are liable to the Company.

231. Plaintiff on behalf of PTC has no adequate remedy at law.

THIRD CLAIM

Against Individual Defendants for Unjust Enrichment

232. Plaintiff incorporates by reference and re-alleges each and every allegation set forth above, as though fully set forth herein.

233. By their wrongful acts, violations of law, and false and misleading statements and omissions of material fact that they made and/or caused to be made, the Individual Defendants were unjustly enriched at the expense of, and to the detriment of, PTC.

234. The Individual Defendants either benefitted financially from the improper conduct and their engaging in lucrative insider transactions tied to the false and misleading statements, or received bonuses, stock options, or similar compensation from PTC that was tied to the performance or artificially inflated valuation of PTC, or received compensation that was unjust in light of the Individual Defendants' bad faith conduct.

235. Plaintiff, as a shareholder and a representative of PTC, seeks restitution from the Individual Defendants and seeks an order from this Court disgorging all profits, including from insider transactions, benefits, and other compensation, including any performance-based or valuation-based compensation, obtained by the Individual Defendants due to their wrongful conduct and breach of their fiduciary and contractual duties.

236. Plaintiff on behalf of PTC has no adequate remedy at law.

FOURTH CLAIM

Against Individual Defendants for Abuse of Control

237. Plaintiff incorporates by reference and re-alleges each and every allegation set forth above, as though fully set forth herein.

238. The Individual Defendants' misconduct alleged herein constituted an abuse of their ability to control and influence PTC, for which they are legally responsible.

239. As a direct and proximate result of the Individual Defendants' abuse of control, PTC has sustained significant damages. As a direct and proximate result of the Individual Defendants' breaches of their fiduciary obligations of candor, good faith, and loyalty, PTC has sustained and continues to sustain significant damages. As a result of the misconduct alleged herein, the Individual Defendants are liable to the Company.

240. Plaintiff on behalf of PTC has no adequate remedy at law.

FIFTH CLAIM

Against Individual Defendants for Gross Mismanagement

241. Plaintiff incorporates by reference and re-alleges each and every allegation set forth above, as though fully set forth herein.

242. By their actions alleged herein, the Individual Defendants, either directly or through aiding and abetting, abandoned and abdicated their responsibilities and fiduciary duties with regard to prudently managing the assets and business of PTC in a manner consistent with the operations of a publicly-held corporation.

243. As a direct and proximate result of the Individual Defendants' gross mismanagement and breaches of duty alleged herein, PTC has sustained and will continue to sustain significant damages.

244. As a result of the misconduct and breaches of duty alleged herein, the Individual Defendants are liable to the Company.

245. Plaintiff on behalf of PTC has no adequate remedy at law.

SIXTH CLAIM

Against Individual Defendants for Waste of Corporate Assets

246. Plaintiff incorporates by reference and re-alleges each and every allegation set forth above, as though fully set forth herein.

247. The Individual Defendants caused the Company to pay themselves excessive salaries, bonuses, fees, and stock grants to the detriment of the shareholders and the Company.

248. As a result of the foregoing, and by failing to properly consider the interests of the Company and its public shareholders, Defendants have caused PTC to waste valuable corporate assets, to incur many millions of dollars of legal liability and/or costs to defend unlawful actions,

to engage in internal investigations, and to lose financing from investors and business from future customers who no longer trust the Company and its products.

249. As a result of the waste of corporate assets, the Individual Defendants are each liable to the Company.

250. Plaintiff on behalf of PTC has no adequate remedy at law.

PRAYER FOR RELIEF

FOR THESE REASONS, Plaintiff demands judgment in the Company's favor against all Individual Defendants as follows:

(a) Declaring that Plaintiff may maintain this action on behalf of PTC, and that Plaintiff is an adequate representative of the Company;

(b) Declaring that the Individual Defendants have breached and/or aided and abetted the breach of their fiduciary duties to PTC;

(c) Determining and awarding to PTC the damages sustained by it as a result of the violations set forth above from each of the Individual Defendants, jointly and severally, together with pre-judgment and post-judgment interest thereon;

(d) Directing PTC and the Individual Defendants to take all necessary actions to reform and improve its corporate governance and internal procedures to comply with applicable laws and to protect PTC and its shareholders from a repeat of the damaging events described herein, including, but not limited to, putting forward for shareholder vote the following resolutions for amendments to the Company's Bylaws or Articles of Incorporation and the following actions as may be necessary to ensure proper corporate governance policies:

1. a proposal to strengthen the Board's supervision of operations and develop and implement procedures for greater shareholder input into the policies and guidelines of the board;

2. a provision to permit the shareholders of PTC to nominate at least four candidates for election to the board; and

3. a proposal to ensure the establishment of effective oversight of compliance with applicable laws, rules, and regulations.

(e) Awarding PTC restitution from Individual Defendants, and each of them;

(f) Awarding Plaintiff the costs and disbursements of this action, including reasonable attorneys' and experts' fees, costs, and expenses; and

(g) Granting such other and further relief as the Court may deem just and proper.

Dated: October 10, 2017

Respectfully submitted,

THE ROSEN LAW FIRM, P.A.

/s/ Laurence M. Rosen

Laurence M. Rosen
609 W. South Orange Avenue, Suite 2P
South Orange, NJ 07079
Telephone: (973) 313-1887
Facsimile: (973) 833-0399
Email: lrosen@rosenlegal.com

Attorneys for Plaintiff

VERIFICATION

I, Ned Kim, am the plaintiff in the within action. I have read the foregoing complaint and know the contents thereof. The allegations of the complaint are true of my personal knowledge, except as to the matters therein stated to be alleged on information and belief, and as to those matters I believe them to be true.

I declare under penalty of perjury under the laws of the United States that the foregoing is true and correct. Executed this 28 day of September, 2017.

Ned Kim